BEFORE THE

INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
AND THE APPLICATION REVIEW SUBCOMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT

REGULAR MEETING

LOCATION: CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
1999 HARRISON STREET, SUITE 1650
OAKLAND, CALIFORNIA

DATE: DECEMBER 14, 2017
9 A.M.

REPORTER: BETH C. DRAIN, CSR
CA CSR. NO. 7152

FILE NO.: 2017-26

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I N D E X (CONT'D.)

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THURSDAY, DECEMBER 14, 2017; 9 A.M.

CHAIRMAN THOMAS: GOOD MORNING, EVERYBODY, FROM BEAUTIFUL, CRISP OAKLAND. THIS IS THE DECEMBER AND FINAL MEETING OF THE ICOC AND THE APPLICATION REVIEW SUBCOMMITTEE. I'D LIKE TO WELCOME EVERYBODY HERE. BEFORE WE GET GOING, A COUPLE OF LOGISTICAL ISSUES. FOR MEMBERS OF THE BOARD, THE MICS ARE PUSH AND TALK. YOU DON'T HAVE TO HOLD IT DOWN, BUT YOU DO NEED TO PUSH.

SECONDLY, I KNOW WE HAVE A NUMBER OF PEOPLE HERE WHO ARE INTERESTED IN GIVING PUBLIC COMMENT. I WANT TO MAKE SURE THAT EVERYBODY UNDERSTANDS THE TIMING OF PUBLIC COMMENT. IF THERE IS AN AGENDIZED TOPIC THAT IS UNDER DISCUSSION AT THAT PARTICULAR MOMENT THAT YOU HAVE AN INTEREST IN DISCUSSING, THAT IS WHEN YOU SHOULD GIVE YOUR PUBLIC COMMENT. IF YOUR COMMENTS DO NOT PERTAIN TO A PARTICULAR MOTION ON THE FLOOR, I WOULD ASK THAT YOU PLEASE HOLD THOSE COMMENTS TILL THE GENERAL PUBLIC COMMENT AT THE END OF THE MEETING, AT WHICH TIME YOU WILL BE HEARD TO THE FULL EXTENT THAT WE CAN PROCEDURALLY HERE. THANK YOU FOR THAT.

WITH THAT, COULD WE HAVE THE PLEDGE OF ALLEGIANCE PLEASE, MARIA.

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(THE PLEDGE OF ALLEGIANCE.)

CHAIRMAN THOMAS: WE'RE GOING TO GO FORTHWITH TO THE PRESIDENT'S REPORT. DR. MILLAN.

MS. BONNEVILLE: WE NEED TO TAKE ROLL.

CHAIRMAN THOMAS: ROLL CALL. THANK YOU, MARIA.

MS. BONNEVILLE: THOSE OF YOU ON THE PHONE, IF YOU COULD UNMUTE AND ALSO IF YOU COULD LET ME KNOW IF THERE ARE MEMBERS OF THE PUBLIC AT YOUR LOCATION WHEN I CALL ROLL.

GEORGE BLUMENTHAL.

DR. BLUMENTHAL: HERE.

MS. BONNEVILLE: LARS BERGLUND.

DR. BERGLUND: HERE.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: HERE.

MS. BONNEVILLE: DEBORAH DEAS. JACK DIXON.

DR. DIXON: HERE. NO OUTSIDE PEOPLE.

I'LL JUST ADD ONE FOOTNOTE. I'M STEPPING IN FOR DAVID BRENNER WHO LOST HIS MOTHER THIS PAST WEEK.

MS. BONNEVILLE: SORRY TO HEAR THAT.

ANNE-MARIE DULIEGE.

DR. DULIEGE: HERE.

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MS. BONNEVILLE: HOWARD FEDEROFF. JUDY GASSON.

DR. GASSON: HERE.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

DR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. BERT LUBIN.

DR. LUBIN: HERE.

MS. BONNEVILLE: LINDA MALKAS.

DR. MALKAS: HERE.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: HERE.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: HERE.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: HERE.

MS. BONNEVILLE: JOE PANETTA.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: HERE.

MS. BONNEVILLE: ROBERT QUINT. AL ROWLETT.

MR. ROWLETT: HERE.

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MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: HERE.

MS. BONNEVILLE: OSWALD STEWARD.

DR. STEWARD: HERE.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: HERE.

MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: HERE. NO PUBLIC.

MS. BONNEVILLE: DIANE WINOKUR.

CHAIRMAN THOMAS: THANK YOU, MARIA.

WE HAVE QUITE A FULL AGENDA TODAY. BEFORE WE GET GOING HERE, SENATOR TORRES WOULD LIKE TO MAKE A STATEMENT.

MR. TORRES: YES, MR. CHAIRMAN AND MEMBERS. ON TUESDAY MORNING, I LOST A FRIEND WHO I'VE KNOWN SINCE 1978 AND WHO WAS A GREAT MAYOR OF THE CITY AND COUNTY OF SAN FRANCISCO. ED LEE. ED AND I FIRST MET WHEN HE WAS A CIVIL RIGHTS LAWYER IN LOS ANGELES AND SAN FRANCISCO AS WE FOUGHT FOR ASIAN AMERICAN AND PACIFIC ISLANDER RIGHTS THROUGHOUT THE STATE.

FROM THE VERY BEGINNING, HE WAS A TREMENDOUS SUPPORTER OF OUR CAUSE. AS BOB KLEIN

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WELL KNOWS, WHO'S HERE IN THE AUDIENCE, IT WAS ED LEE WHO REALLY SHEPHERDED US AFTER GAVIN NEWSOM LEFT US WITH A FREE LEASE OF TEN YEARS. AND ED LEE CONTINUED THAT LEGACY. AND, OF COURSE, HE WAS HEARTBROKEN WHEN WE LEFT FOR OAKLAND, BUT THE HIGH RENTS COULDN'T KEEP US THERE. BUT ED HAS GARNERED THE SUPPORT AND THE ADMIRATION OF SO MANY.

SO I WOULD ASK VERY HUMBLY THAT WE ADJOURN TODAY'S MEETING IN HIS MEMORY.

CHAIRMAN THOMAS: THANK YOU, MR. SENATOR.

SUPERVISOR SHEEHY: I WOULD ECHO THAT. A TREMENDOUS LOSS FOR THE CITIZENS OF SAN FRANCISCO AND THE WHOLE BAY AREA REGION AND THE STATE. AND THE WHOLE CITY IS REELING, BUT WE'RE STILL FUNCTIONING. BUT THANK YOU FOR YOUR WORDS, ART.

CHAIRMAN THOMAS: THANK YOU, MR. SENATOR AND MR. SUPERVISOR.

WE WILL PROCEED NOW TO ITEM NO. 4, WHICH IS PRESIDENT'S REPORT. DR. MILLAN.

DR. MILLAN: MEMBERS OF THE PUBLIC AND COLLEAGUES, I'LL BE PRESENTING A PRESIDENT'S REPORT TODAY. AND I'LL START OFF WITH OUR MISSION AS THE REASON WE'RE HERE, AND THE REASON WE'RE ASSEMBLED TODAY IS TO ACCELERATE STEM CELL TREATMENTS TO PATIENTS WITH UNMET MEDICAL NEEDS. IN TODAY'S

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PRESIDENT'S REPORT, I'LL COVER SOME 2017 HIGHLIGHTS AND AN UPDATE ON OUR PROGRESS TOWARD OUR FIVE-YEAR STRATEGIC PLAN. I WILL BE GIVING AN UPDATE ON OUR RESEARCH BUDGET. AND ON BEHALF OF THE CIRM TEAM WILL BE BRINGING A PROPOSAL AND REQUESTED ACTION FROM THIS BOARD FOR TWO ITEMS: THE CLIN AWARD CAP BUDGET AS WELL AS THE 2018 BUDGET FOR APPROVAL.

SO IN TERMS OF 2017, I THINK MANY OF US WILL SEE 2017 AS A VERY REMARKABLE YEAR FOR THE FIELD OF CELL THERAPIES. THERE HAVE BEEN TWO WHAT'S CALLED LIVING CELLS OR GENE-MODIFIED CELL THERAPIES APPROVED NOW BY THE FDA. ON AUGUST 30TH NOVARTIS' T-CELL CAR-T THERAPY WAS APPROVED FOR AML, AND SHORTLY THEREAFTER A DEAL BETWEEN KITE AND GILEAD TRANSPIRED, AND THEN THAT PRODUCT WAS ANOTHER CAR-T THERAPY, WHICH IS A GENE-MODIFIED CELL THERAPY FOR THE TREATMENT OF B-CELL LYMPHOMA, WAS ALSO APPROVED BY THE FDA.

SO THAT IS FOR THE FIELD KIND OF A MARK THAT THE FIELD IS MATURING. THERE'S BEEN A LOT OF ENSUING CONVERSATIONS ABOUT REIMBURSEMENT AND DOWNSTREAM CONSIDERATIONS FOR ADOPTION AND PRACTICALLY HOW DO WE GET THESE TO THE PATIENTS IN NEED. SO THAT IS GOING TO BE THE NEXT PHASE OF CHALLENGES FOR THAT PARTICULAR PRODUCT, BUT A VERY

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IMPORTANT CONVERSATION WHICH WILL ALSO INFORM US FOR THESE CELL THERAPIES AND REGENERATIVE MEDICINE FIELD IN GENERAL.

THIS YEAR WAS ALSO MARKED BY THE LAUNCH OF THE 21ST CENTURY CURES ACT THAT WAS PASSED BY CONGRESS IN DECEMBER 2016 THAT LED TO CREATION OF A NEW, EXPEDITED PATHWAY IN THE FDA CALLED THE RMAT, THE REGENERATIVE MEDICINE ADVANCED THERAPIES, EXPEDITED PATHWAY. AND I'LL GO INTO THAT IN A LITTLE BIT MORE DETAIL.

I THINK IT MARKS TWO THINGS. ONE, IT'S A RECOGNITION ON THE FEDERAL LEVEL AND BY CONGRESS THAT THE PROMISE OF THE REGENERATIVE STEM CELL FIELD AND ITS IMPORTANCE IN MEDICINE AND THE FUTURE OF HEALTHCARE. AND THE OTHER ITEM THAT I WILL GIVING AN UPDATE ON IS OUR CONTINUED CONVERSATIONS AND COLLABORATIVE ACTIVITIES WITH THE NIH WHICH HAS ENSUED SINCE OUR INVITED VISIT BY FRANCES COLLINS' OFFICE AND THE INSTITUTE HEADS OF THE NIH IN JUNE. WE'VE HAD SOME FOLLOW-UP ACTIVITIES SINCE THEN.

SO, FIRST, I'D LIKE TO JUST GIVE AN UPDATE ON WHAT THE RMAT EXPEDITED REGULATORY PATHWAY IS. THE RMAT EXPEDITED REGULATORY PATHWAY WAS CREATED BY THE 21ST CENTURY CURES ACT, AND THE FDA HAS FULLY COMMITTED TO THIS. THE COMMISSIONER, SCOTT

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GOTTLIEB, HAS RESOURCED THE OFFICE OF TISSUE AND ADVANCED THERAPIES TO BE ABLE TO PROCESS IN A MOST EFFICIENT WAY APPLICATIONS FOR THIS EXPEDITED PATHWAY. AND I'M PLEASED TO SAY THAT CIRM HAS BEEN AT THE FOREFRONT OF THE FIRST THREE OF THESE EXPEDITED DESIGNATIONS. TWO OF THEM WERE CIRM PROGRAMS. AND CURRENTLY THERE ARE ELEVEN SO FAR THIS YEAR. AND THAT'S THE MOST UP-TO-DATE DATA FROM THE FDA REPORT AT THE MEETING WE ATTENDED LAST WEEK.

OF THOSE ELEVEN, THREE OF THEM ARE CIRM PROGRAMS. SO, AGAIN, IT'S AN INDICATION OF HOW CIRM AND ITS STAKEHOLDERS JUST CONTINUE TO BE IN THE FOREFRONT OF THIS EFFORT AND DRIVE THIS FORWARD.

THE RMAT ALLOWS FOR A NIMBLE PROCESS, FREQUENT CONVERSATIONS WITH FDA, KIND OF A REAL-TIME EVALUATION OF WHERE THE DATA IS. IT'S A WAY THAT THEY CAN BREAK AWAY FROM THE TRADITIONAL WAY OF LOOKING AT DRUG DEVELOPMENT, WHICH WAS MORE RELEVANT FOR SMALL MOLECULES THAN MAYBE TRADITIONAL BIOLOGICS, THEREFORE, REALLY LOOK AT WHAT THIS PRODUCT IS, WHAT CONSIDERATIONS IN TERMS OF WHAT REAL EFFECTS THAT ARE BENEFICIAL TO THE PATIENTS. THEY COULD LOOK AT WELCOME SURROGATE MARKERS, WHICH IN THE PAST IS REALLY TOUGH TO GET THROUGH. SO IT'S VERY EXCITING.

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OUR TEAM HAS BEEN VERY INTERACTIVE WITH THE LEADERSHIP OF THE FDA AND WILL CONTINUE THOSE CONVERSATIONS TO DETERMINE THE BEST WAY TO HAVE OUR PROGRAMS UTILIZE THE EXPEDITED PATHWAY AS WELL AS, IN GENERAL, INFORM EACH OTHER ALONG THE WAY IN TERMS OF HOW BEST TO DEVELOP THESE PRODUCTS.

THE THREE PROGRAMS THAT RECEIVED THE RMAT EARLY ON IS HUMACYTE, WHICH IS A BIOLOGIC VASCULAR GRAFT FOR DIALYSIS ACCESS AND END STAGE RENAL DISEASE; JCYTE'S AMD TRIAL, A CELL THERAPY TRIAL; ASTERIAS' CELL THERAPY REPLACEMENT AND REPAIR TRIAL FOR SPINAL CORD INJURY.

IN TERMS OF THE CIRM-NIH PARTNERSHIP, AS WE REPORTED EARLIER IN THE YEAR, OUR TEAM MET WITH THE NIH IN JUNE. WE WERE INVITED THERE BECAUSE THEY WERE EXPLORING WAYS THAT THE NIH COULD REALLY GEAR UP TO FULFILL THE ASPIRATIONS AND REQUIREMENTS OF THE 21ST CENTURY CURES ACT AND FURTHER REGENERATIVE MEDICINE RESEARCH. THE NIH WAS VERY MUCH IMPRESSED BY THE CIRM PROCESSES AND THE WAY THAT WE WERE ABLE TO GET OUR PROGRAMS TO LATE DEVELOPMENT AND INTO THE CLINICAL TRIALS.

FROM THAT WE HAD SERIALLY MULTIPLE INVITATIONS TO GO BACK AND VARIOUS WORKSHOPS TOPIC RELATED, OPERATION OR OTHERWISE, AND IT'S LED TO

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SEVERAL KEY, CONCRETE OUTCOMES. ONE IS THAT CIRM WAS INVITED TO PARTICIPATE IN THE NIH-FDA REGENERATIVE MEDICINE INNOVATION WORKSHOP LAST WEEK. IT WAS AN EXTREMELY USEFUL WORKSHOP WHERE INVESTIGATORS IN THE FIELD, MANY, MANY REPRESENTATIVES FROM THE FDA, AND ALL OF THE INSTITUTE HEADS OF THE NIH ASSEMBLED IN A ROOM ALONG WITH INVESTIGATORS WISHING TO PUSH THEIR PROGRAMS INTO THE CLINICS INTO DEVELOPMENT.

AND THERE WERE SOME INTERESTING THINGS THAT CAME OUT. FIRST OF ALL, THE NIH HAS, REALLY ONLY FOR THIS REGENERATIVE MEDICINE INNOVATION FUND, HAS $30 MILLION IN FUNDING TO SUPPORT SUCH EFFORTS OVER THE NEXT FIVE YEARS. AT LEAST THAT'S WHAT'S CURRENTLY COMMITTED UNDER THE CURES ACT. AND THEY CURRENTLY ARE FOCUSING ON ADULT STEM CELLS. THEY REALIZE THAT CIRM WILL CONTINUE TO FUND THE OTHER TYPES OF STEM CELLS, BUT THEY CURRENTLY WILL FOCUS ON ADULT STEM CELLS.

ANOTHER THING THAT THE INVESTIGATORS POINTED OUT WAS THAT IT WAS VERY, VERY DIFFICULT TO FIGURE OUT A WAY TO ACTUALLY GET THEIR TRANSLATIONAL RESEARCH EVEN FUNDED, THE SO-CALLED VALLEY OF DEATH. AND THAT STILL PERSISTS. AND THE RESPONSE WAS KEEP TRYING AND IT MIGHT COME THROUGH. BUT I THINK THAT

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THE NIH IS GOING TO BE RECEPTIVE TO HOW DO YOU DO THIS. AND THEY HAVE BEEN HAVING CONVERSATIONS WITH US, AND WE'VE ACTUALLY BEEN WORKING WITH THEM ON THIS. BUT IT'S REALLY KEY BECAUSE THAT'S WHAT CIRM BRINGS TO THE TABLE THAT NOBODY ELSE DOES. FUND THE EARLY TRANSLATIONAL RESEARCH AND THE EARLY STAGE CLINICAL TRIALS. FUND THEM AT A TIME WHEN OTHERS WON'T YET FUND THEM, SO-CALLED DERISK THE PROGRAMS, SO THAT THEY CAN GATHER INFORMATION AND DATA THAT WILL ALLOW OTHERS TO LOOK AT IT, AND THEN COME IN, WHETHER IT BE PHARMA OR OTHER INVESTORS, AND BRING THIS FORWARD AND SUPPORT IT DOWNSTREAM.

ANOTHER OUTCOME OF THIS -- AND ARLENE CHIU, I SEE, HAS ATTENDED FROM THE CITY OF HOPE -- WAS A JOINT CIRM-NIH SITE VISIT TO THE CITY OF HOPE BECAUSE THE NIH IS EMBARKING UPON A SICKLE CELL CURES INITIATIVE. AND THEY VERY MUCH WERE INTERESTED ABOUT OUR CIRM PROGRAMS, BUT ALSO THE INFRASTRUCTURE AND THE INTEGRATED CAPABILITIES WE PUT TOGETHER BECAUSE OF OUR MULTIPLE GRANTEES, BUT INFRASTRUCTURE PROGRAMS AND THE ECOSYSTEM THAT'S BEEN BUILT IN CALIFORNIA. AND BECAUSE OF THAT JOINT SITE VISIT, THE CITY OF HOPE WAS AWARDED ADDITIONAL FUNDS FOR THEIR MANUFACTURE AND PROCESS DEVELOPMENT EFFORTS IN THE GENE THERAPY SPACE.

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ANOTHER KIND OF DOWN IN THE WEEDS KIND OF ON-THE-GROUND WORK THAT'S BEING DONE, PAT OLSON AND GABE THOMPSON HAVE PURSUED WITH NIH HOW SOME OF THEIR MULTICENTER AWARDS CAN BE CRAFTED IN A WAY THAT HAVE MILESTONES AND OUTCOMES THAT WOULD SET THEM UP WELL TO GET INTO THE TRANSLATIONAL STAGE AND LATE DEVELOPMENT STAGE, AGAIN, BECAUSE NIH RECOGNIZED THAT THEY WE WERE ABLE TO BUILD A VERY STRONG LATE DEVELOPMENT PORTFOLIO. AND THAT'S REALLY MOVING CLOSER TO MAYBE BRINGING US MORE TRANSLATIONAL PROJECTS.

SO IN TERMS OF THE NEXT SLIDE, 2017 UPDATE, AGAIN, CIRM UNIQUELY FUNDS THESE FIVE PILLARS: INFRASTRUCTURE, EDUCATION, DISCOVERY, TRANSLATION, AND CLINICAL. AND THIS YEAR WITH MAYBE SOME MODIFICATION BASED ON TODAY'S ICOC CONSIDERATIONS OF GRANTS IN THE DISCOVERY CATEGORY, THESE HAVE BEEN OUR INVESTMENTS IN 2017 INTO THE FIVE PILLARS OF PROGRAMS. $16 MILLION IN INFRASTRUCTURE TO FUND TWO ADDITIONAL ALPHA CLINICS, THE EXPANSION OF THE ALPHA CLINICS NETWORK, A MILLION DOLLARS IN EDUCATION, $45 MILLION IN DISCOVERY, $24 MILLION IN TRANSLATION, AND $213 MILLION IN CLINICAL TO FUND 16 ADDITIONAL NEW CLINICAL TRIALS INTO OUR PORTFOLIO.

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SO YOU WILL RECALL THAT WHEN WE LAUNCHED CIRM 2.0 AND THOSE SYSTEMS, THE IDEA WAS TO CREATE A MORE EFFICIENT ACCELERATING ENGINE. AND WE REPORTED VERY FAVORABLE RESULTS WITH THE LAUNCH OF THIS SYSTEM LAST YEAR. AND I'M PLEASED TO SAY THAT THIS HAS BEEN A DURABLE EFFECT, AND IN 2017 WE CONTINUE WITH PERFORMANCE AS SHOWN IN THIS SCHEMATIC WHERE WE HAVE INCREASED BY 33 PERCENT MORE APPLICATIONS COMING INTO OUR SYSTEM, 75 PERCENT MORE HIGH QUALITY APPLICATIONS BEING RECOMMENDED BY OUR GWG.

AND THE GWG, FOR THOSE WHO PARTICIPATE, REMAIN EXTREMELY RIGOROUS IN THEIR REVIEW OF THESE APPLICATIONS. SO THAT JUST SPEAKS TO THE QUALITY OF APPLICATIONS COMING IN. AND OUR TEAM ACTUALLY IS VERY INVOLVED, NOT THE REVIEW TEAM, OUR SCIENCE OFFICE, SEPARATE FROM THE REVIEW TEAM, ARE VERY INVOLVED WITH THE APPLICANTS SO THAT THEY REALLY ARE READY TO COME IN AND BRING IN THE KEY INFORMATION FOR OUR GRANTS WORKING GROUP TO LOOK AT. SO WE BELIEVE THAT ALSO HAS DRIVEN PERFORMANCE.

AND WE WERE ABLE TO DO THIS WITH 57 PERCENT LOWER COST PER APPLICATION. AND, AGAIN, ACCELERATION, 82 PERCENT LESS TIME TO APPROVAL, AND TIME TO FUNDING IS STILL UNDER A HUNDRED FIFTY DAYS FOR ALL AWARDS.

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AND WHAT HAS THIS LED TO? AGAIN, WE STARTED OFF WITH A MISSION. WHAT THIS HAS LED TO IS WE'VE HAD A TWO-AND-A-HALF-FOLD EXPANSION OF OUR CLINICAL TRIAL PORTFOLIO. WE'VE HAD, BECAUSE OF OUR CLINICAL ADVISORY PANEL, WHICH HAS JUST, IN TERMS OF ACTIVITY, HAS INCREASED TWO- OR THREEFOLD IN TERMS OF NUMBERS OF MEETINGS WE HAVE IN ADVISING AND HELPING OUR APPLICANTS MEET THEIR MILESTONES. WE HAVE 75 PERCENT ON TIME. AND FOR THOSE WHO HAVE RUN CLINICAL TRIALS AND KNOW THE DRUG DEVELOPMENT WORLD, 75 PERCENT ON TIME OR EARLY ON MILESTONES IS PRETTY REMARKABLE.

WE HAVE NOW, I GUESS, THE MOST UP-TO-DATE NUMBERS. SEVEN HUNDRED THREE PATIENTS HAVE BEEN ENROLLED AND TREATED IN CIRM-FUNDED CLINICAL TRIALS TO DATE.

NOW I'LL JUST GIVE AN UPDATE ON WHERE WE ARE IN RELATION TO OUR FIVE-YEAR STRATEGIC PLAN. AS YOU RECALL, THIS BOARD APPROVED OUR FIVE-YEAR STRATEGIC PLAN IN DECEMBER 2015. WE LAUNCHED IT IN JANUARY 2016. AND SO WE ARE NOW ENDING YEAR TWO OF OUR FIVE-YEAR STRATEGIC PLAN.

THE PLAN CENTERED AROUND SIX BIG, BOLD GOALS, AND I'LL JUST GO THROUGH THE SIX GOALS AND WHERE WE ARE ON EACH OF THESE.

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THE FIRST GOAL WAS SO-CALLED DISCOVER, BRING 50 NEW DEVELOPMENT CANDIDATES INTO THE CIRM PIPELINE. IN YEAR TWO WE HAVE BROUGHT IN 24 OF THE TARGET OF 50 NEW CANDIDATES. SO THAT'S AHEAD OF SCHEDULE. TO INCREASE THE PROBABILITY OR THE INCIDENCE OF PROGRAMS MOVING FROM ONE STAGE OF RESEARCH TO THE NEXT STAGE, FROM DISCOVERY TO TRANSLATIONAL, FROM TRANSLATIONAL TO PRECLINICAL, FROM PRECLINICAL TO CLINICAL. AND WE'VE DOUBLED THE INCIDENCE OF PROGRESSION FROM ONE STAGE OF RESEARCH TO THE NEXT.

REFINE IS ENACT A NEW REGULATORY PARADIGM THAT IS APPROPRIATE FOR REGENERATIVE MEDICINE AND STEM CELL THERAPIES. AND AS I MENTIONED EARLY ON IN THE PRESENTATION, CIRM PROGRAMS ACCOUNT NOW FOR 26 PERCENT OF RMAT THAT THE FDA HAS SO FAR AWARDED OR GIVEN TO PROJECTS IN THE U.S.

AND IN TERMS OF ACCELERATE, THE GOAL WAS TO BRING DOWN THE TIME IT TAKES TO DEVELOP A CANDIDATE TO GET IT INTO THE PATIENTS BY HALF. AND IN ORDER TO DO THAT, WE LOOK AT THINGS LIKE DECREASING TIME OF TRANSLATION AND DECREASING TIME TO GET TO IND. SO JUST AS A MEASURE THAT WE CAN CURRENTLY LOOK AT, WE HAVE ALREADY ACHIEVED JUST THIS YEAR ALONE THREE OF OUR PROGRAMS THAT WERE IN

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THE IND-ENABLING STAGE ACHIEVING AN IND. THAT'S BEEN ALLOWED BY THE FDA WITHIN 18 MONTHS. AGAIN, PRETTY REMARKABLE IN TERMS OF TIMELINE.

IN TERMS OF VALIDATE, THIS IS SOMETHING THAT MANY ARE MOST FAMILIAR WITH. THE GOAL WAS TO BRING IN 50 NEW CLINICAL TRIALS INTO THE CIRM PORTFOLIO IN FIVE YEARS. IN YEAR TWO WE ARE AHEAD OF SCHEDULE, AND WE ALREADY HAVE 26 NEW, HIGH QUALITY, WELL-SCORED CLINICAL TRIAL PROJECTS INTO OUR CIRM PORTFOLIO.

AND THEN THE FINAL GOAL, WHICH IS TO INCREASE INDUSTRY PULL AND INCREASE PARTNERSHIP AND INVESTMENT INTO OUR PROGRAMS.

AND IN 2017 SIX INVESTMENTS HAVE BEEN MADE INTO OUR PROGRAM, AND FIVE NEW PARTNERSHIPS OR ACQUISITIONS HAVE TAKEN PLACE WITH THE PROGRAMS THAT WE FUNDED INITIALLY. AND IF YOU SEE ON THE ARROW, THE AMOUNT OF PRIVATE INDUSTRY FUNDING THAT CIRM PROGRAMS HAD OBTAINED, IN 2015 $41 MILLION OF INDUSTRY PARTNERSHIP FUNDING WAS AWARDED TO OUR PORTFOLIO PROGRAMS, IN 2016 125 MILLION, AND THIS YEAR ALONE ALMOST $307 MILLION IN TERMS OF INVESTMENT, WHETHER IT BE SERIES A, B, OR ADDITIONAL PARTNERSHIP DEALS.

SO CIRM DOLLARS ARE BEING LEVERAGED. AND

TO DATE AN ADDITIONAL $1.7 BILLION HAVE COME IN TO SUPPLEMENT WHAT CIRM HAS INVESTED INTO THE PROGRAM IN THE FORM OF $911 MILLION IN CO-FUNDING, EITHER THROUGH THE INSTITUTIONS OR THE COMPANIES WHO HAVE COME IN AND ARE INVOLVED IN THE PROGRAMS THAT CIRM IS FUNDING, $473 MILLION, AS PER THE PREVIOUS SLIDE, IN PARTNERSHIP FUNDING, AND OVER $390 MILLION IN ADDITIONAL GRANT FUNDING OR PHILANTHROPIC FUNDS TO INVESTIGATORS BECAUSE THEY HAD OBTAINED CIRM FUNDING.

SO WITH THAT CONTEXT AND WITH THAT UPDATE, I'D LIKE TO MOVE TO AGENDA ITEM NO. 5 AND PROVIDE THE BOARD A CIRM BUDGET UPDATE. BEFORE I PROCEED, SHOULD I TAKE QUESTIONS, CHAIRMAN THOMAS?

CHAIRMAN THOMAS: SURE, IF THERE ARE ANY. THANK YOU. I WOULD JUST LIKE TO, IF THERE AREN'T ANY QUESTIONS, JUST SAY I CONGRATULATE DR. MILLAN AND ALL MEMBERS OF THE TEAM HERE. ANYBODY WHO HEARS THAT REPORT CANNOT THINK ANYTHING BUT THAT THINGS ARE REALLY MOVING ON ALL CYLINDERS AND ARE DRAMATICALLY ACCELERATING THE FIELD ACROSS MANY DIFFERENT INDICATIONS. SO I THINK THAT EVERYBODY WHO'S AFFILIATED WITH CIRM, AND THAT INCLUDES ALL OF OUR STAKEHOLDERS, MANY OF WHICH ARE HERE, SHOULD FEEL VERY, VERY PROUD OF WHAT WE ALL COLLECTIVELY

ARE DOING AND WHAT OUR QUEST IS BECOMING HERE. SO THANK YOU.

DR. MILLAN: THANK YOU VERY MUCH FOR THOSE COMMENTS.

AGENDA ITEM NO. 5, I'LL START WITH A BUDGET UPDATE, AND THIS WILL LEAD TO TWO ACTIONS THAT WE WOULD PROPOSE FOR THE BOARD.

SO AS OF TODAY, OUR ESTIMATE FOR OUR RESEARCH BUDGET FOR BEGINNING JANUARY 1, 2018, IS THE REMAINING APPROXIMATELY $335 MILLION IN THE RESEARCH BUCKET AND $48 MILLION IN THE ADMINISTRATION BUCKET.

SO, FIRST, I'D LIKE TO JUST GIVE AN UPDATE ON THE BUDGET WITH RESPECT TO OUR STRATEGIC PLAN. WHEN WE LAUNCHED OUR STRATEGIC PLAN IN JANUARY 2016, WE HAD AN $890 MILLION BUDGET. WHAT WE'VE EXPERIENCED IN THE ENSUING TWO YEARS IS AN INCREDIBLE AND UNPRECEDENTED SUCCESS OF THE CLINICAL PROGRAM WHICH HAS LED TO A FASTER THAN EXPECTED EXPENDITURE OF THIS BUDGET. WE ACTUALLY EXPECT THAT, WITH WHAT WE CURRENTLY KNOW IS IN THE PIPELINE AND WHAT OUR CURRENT PERFORMANCE IS, WE ESTIMATE THAT THE LAST AWARDS WILL BE IN THE END OF 2019, AND THIS DIFFERS FROM OUR ORIGINAL PROJECTION OF MID-2020.

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THE SECOND KIND OF FORECAST FOR THE STRATEGIC PLAN IS THAT $440 MILLION WOULD BE EXPENDED ON CLINICAL PROGRAMS AND WOULD BE SUFFICIENT TO FUND 50 NEW CLINICAL TRIALS. AS OF TODAY, $300 MILLION HAS ALREADY BEEN EXPENDED ON 26 CLINICAL TRIALS AND 9 CLIN1S, WHICH ARE IND-ENABLING WORK TO GET TO THE IND AND TO CLINICAL TRIALS.

WHAT WE'VE SEEN IS THAT THE AVERAGE CLINICAL TRIAL AWARD HAS INCREASED FROM $10.9 MILLION IN THE 2015-16 PERIOD TO $12.1 MILLION IN 2017.

THE THIRD UPDATE IS THAT WHEN WE LAUNCHED THE STRATEGIC PLAN, WE HAD THOUGHT THAT THE ADMINISTRATION BUDGET WOULD BE SOMETHING THAT WOULD RUN OUT BEFORE THE RESEARCH BUDGET, BUT WHAT WE'RE SEEING NOW IS THAT THE RESEARCH BUDGET COULD BE FULLY EXPENDED BEFORE THE ADMINISTRATION BUDGET WHERE THE ADMINISTRATION BUDGET CAN CARRY US BEYOND 2020; WHEREAS, THE RESEARCH ALLOCATIONS PROBABLY WOULD END BY THE END OF 2019.

SO JUST AS AN UPDATE FOR THIS, I GAVE THIS UPDATE IN THE SCHEMATIC, BUT JUST IN TERMS OF WHAT WAS BUDGETED FOR 2017, THIS BOARD APPROVED $329 MILLION TO FUND RESEARCH PROGRAMS, AND THIS INCLUDED THE PROPOSED $75 MILLION FOR ATP3. AS YOU KNOW, THE

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ATP3 PROGRAM DID NOT GO TO REVIEW. SO GIVEN THAT, THE REST OF THOSE FUNDS WERE USED TO FUND THE CLINICAL PROGRAMS. AND THE ESTIMATED TOTAL 2017 RESEARCH AWARDS IS $300 MILLION. AGAIN, THAT MAY VARY DEPENDING ON TODAY'S BOARD ACTION ON THE PROPOSED DISCOVERY PROGRAM.

SO BY YEAR-END IN TERMS OF IF YOU LOOK AT IT COMMITTED AND UNCOMMITTED, WE WILL HAVE $269 MILLION REMAINING FROM PROP 71 FUNDS TO FUND RESEARCH, BUT WE HAVE HAD FUTURE RECOVERY OF FUNDS, UNEXPENDED FUNDS THAT GET RETURNED TO CIRM. AND BASED ON A CONSERVATIVE ESTIMATE, WE BELIEVE THAT IN TOTAL WE'LL HAVE $335 MILLION IN THE RESEARCH BUDGET OVER THE ENSUING THREE YEARS.

SO OUR TEAM HAS ENGAGED IN A VERY DEEP EXERCISE IN LOOKING AT VARIOUS BUDGET SCENARIOS AND BUDGET PLANNING EXERCISES THAT LED TO OUR PRESENTATION OF THESE SCENARIOS AND CONSIDERATIONS TO THE JOINT SCIENCE AND TRANSITION SUBCOMMITTEE IN NOVEMBER. AND THE BUDGET THAT WILL BE PRESENTED TO YOU TODAY WAS INFORMED BY THAT MEETING AS WELL AS WITH THE OUTCOME OF THAT EXERCISE THAT WE'VE BEEN ENGAGED IN SINCE JUNE OF THIS YEAR.

THE OPERATING PRINCIPLES BEHIND OUR BUDGET PLANNING IS THAT WE REMAIN COMMITTED TO EXECUTING ON

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THE FIVE-YEAR STRATEGIC PLAN. WE THINK IT'S A STRONG PLAN. WE THINK IT'S GIVING GREAT RESULTS. WE THINK IT'S PUSHING THE MISSION. AND I THINK THERE'S GENERAL AGREEMENT TO THAT. THAT THERE IS A CRITICAL PERSONNEL LEVEL THAT'S REQUIRED TO EXECUTE ON THE STRATEGIC PLAN WHILE MAINTAINING THAT VERY EFFICIENT ACCELERATING ENGINE THAT YOU SAW EARLIER. AND THAT IT'S ESSENTIAL TO PRESERVE CIRM'S VALUE PROPOSITION. THE VALUE PROPOSITION IS THE ABILITY TO FUND ITS FULL COMPLEMENT OF PROGRAMS THAT FILLS THE PIPELINE ALL THE WAY FROM THE EARLY RESEARCH ALL THE WAY TO CLINICAL TRIALS.

AND THE OTHER PORTION OF THE VALUE PROPOSITION IS THE CIRM PIECE, THE HUMAN PIECE, WHAT THE CIRM ORGANIZATION BRINGS TO AUGMENT, NOT JUST OUR INVESTMENT AND THE AWARDS, BUT BRINGING IT ALTOGETHER, INFRASTRUCTURE, COORDINATION.

SO WITH $335 MILLION LEFT IN RESEARCH FOR THE LAST AWARDS TO BE AWARDED IN Q4 OF 2019, WHAT WE HAVE FOUND IS THAT WITH THE CURRENT CLIN AWARD, WITH A BUDGET CAP OF UP TO $20 MILLION FOR CLIN AWARDS, WE WOULD ONLY HAVE ENOUGH LEFT IN THE RESEARCH BUDGET TO FUND THE REMAINING TRIALS TO GET US TO THE GOAL OF 50 NEW CLINICAL TRIALS. THERE WOULD NOT BE ENOUGH FUNDING TO FUND THE DISCOVERY AND TRANSLATION

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PROGRAMS, FOR INSTANCE.

WHAT WE'RE BRINGING FORWARD TO THE BOARD, AND I'LL GIVE YOU MORE OF A BACKGROUND AND MORE DETAIL WHAT THE ACTUAL PROPOSAL IS, WE'RE PROPOSING TO YOU TODAY THAT WE REDUCE THE CLIN AWARD CAP AS PER THE PROPOSAL I'M ABOUT TO GET INTO. THAT WOULD GENERATE APPROXIMATELY $68 MILLION IN SAVINGS, AND THAT WOULD BE ENOUGH TO FUND ADDITIONAL DISC AND TRAN PROGRAMS.

THIS SLIDE JUST SPEAKS TO WHAT THE CURRENT CLIN AWARD CAP IS, WHICH IS ACROSS THE BOARD FROM IND-ENABLING STUDIES, PHASE 1, PHASE 2, PHASE 3 CLINICAL TRIALS, UP TO A $20 MILLION BUDGET FOR ANY OF THESE PROGRAMS. THAT'S A HOLDOVER FROM THE DISEASE TEAM MODEL, THE EARLIER FUNDING MECHANISM OF CIRM. THE THING IS THE DISEASE TEAM AWARDS FUNDED THE WHOLE HOST OF ACTIVITIES THAT ARE CURRENTLY COVERED UNDER DISTINCT AND SEPARATE RESEARCH PROGRAMS NOW: THE TRAN THAT GETS TO THE PRE-IND, THE CLIN1 THAT GETS TO THE IND, AND THE CLIN2 THAT EXECUTES ON THE TRIAL. SO THOSE ARE DISTINCT ACTIVITIES WITH DISTINCT BUDGETS. WE'RE, THEREFORE, PROPOSING THAT THE AWARD CAP SHOULD BE ADJUSTED ACCORDINGLY IN THE FOLLOWING WAY. AND JUST AS A COMPARISON, IN THE MIDDLE ROW IS THE AVERAGE AWARD

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AMOUNT FOR EACH OF THESE CATEGORIES. AND I CAN READ THEM ACROSS BRIEFLY. FOR CLIN1S, AVERAGE AWARD SIZE FOR 2017 IS 4.9 MILLION, PHASE 1 OR PHASE 2. PHASE 1, 1/2 IS $10 MILLION. PHASE 2 15 AND PHASE 3 16.7.

WE'RE PROPOSING THE REVISED AWARD CAPS AS SHOWN IN THE LOWEST COLUMN HIGHLIGHTED IN YELLOW WITH A $6 MILLION AWARD CAP FOR CLIN1 FOR NON-PROFITS AND 4 MILLION FOR FOR-PROFITS. THE DIFFERENCE IS BECAUSE FOR-PROFIT ORGANIZATIONS ARE REQUIRED TO COME IN WITH 20 PERCENT CO-FUNDING. AND SO THAT WILL JUST BRING THEM TO THE SAME TOTAL AWARD AMOUNT OR TOTAL BUDGET.

FOR PHASE 1 AND PHASE 1/2, WE'RE PROPOSING $12 MILLION FOR A NONPROFIT AND 8 MILLION FOR FOR-PROFIT. AGAIN, THE DIFFERENCE IS THAT FOR-PROFIT ORGANIZATIONS FOR PHASE 1 ARE REQUIRED TO BRING IN 30 PERCENT CO-FUNDING; WHEREAS, NON-PROFITS HAVE NO CO-FUNDING FOR PHASE 1 OR PHASE 1/2S.

FOR PHASE 2 AND PHASE 3 AWARDS, FOR-PROFIT AND NONPROFIT HAVE THE SAME REQUIREMENT FOR CO-FUNDING, 40 PERCENT AND 50 PERCENT RESPECTIVELY. AND WE'RE PROPOSING A $15 MILLION AWARD CAP FOR PHASE 2 TRIALS AND $10 MILLION FOR PHASE 3.

ONE OBVIOUS THING IS THAT PHASE 3 TRIALS ARE MORE EXPENSIVE. AND WHY IS OUR AWARD CAP,

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THEREFORE, LOWER FOR PHASE 3 TRIALS? THE RATIONALE BEHIND THIS IS THAT BY PHASE 3 THESE INVESTIGATORS WILL HAVE ALREADY CONDUCTED WORK THAT BROUGHT IN CLINICAL DATA. THEY SHOULD ALREADY BE IN A POSITION TO GAIN EXTERNAL PARTNERSHIPS AND INVESTMENT INTO THIS PROGRAM. CIRM IS NOT MEANT TO TAKE ALL OF THESE ALL THE WAY TO COMMERCIALIZATION. AND, THEREFORE, THE STRONG PROGRAMS THAT MERIT GOING TO PHASE 3, WE BELIEVE, SHOULD BE ABLE TO BRING IN THEIR OWN INVESTMENTS.

NEXT SLIDE IS JUST WE WERE ASKED -- OKAY. THAT WAS THE AVERAGE AWARD SIZE. WHAT WAS THE MEDIAN, AND WHAT'S THE RANGE OF AWARDS? SO THIS SLIDE JUST REPRESENTS WHAT THE MINIMUM AND MAXIMUM ARE. AND THE BOTTOM PART OF THE RECTANGLE IS THE MINIMUM, AND THE MAXIMUM IS THE UPPER PART OF THE RECTANGLE OF THE BAR GRAPH FOR EACH OF THESE TYPES OF AWARDS. THE STAR IS THE AVERAGE AWARD SIZE FOR A GIVEN STAGE OF PROGRAM, AND THE MEDIAN IS REPRESENTED IN THE HORIZONTAL LINE.

SO FOR THE MOST PART, THE AVERAGE THAT I PRESENTED IN THE PREVIOUS CHART WAS EITHER CLOSE TO OR EVEN ABOVE WHAT THE MEDIAN WAS. THERE'S SOME QUESTIONS I THINK.

CHAIRMAN THOMAS: SO DR. STEWARD.

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DR. STEWARD: COULD YOU, AS YOU'RE TALKING, MAYBE REFER US TO THIS SPREADSHEET THAT RELATES TO THESE BAR GRAPHS? DOES THAT MAKE SENSE? THERE'S A LOT TO DIGEST HERE, AND IT WOULD JUST BE MAYBE USEFUL IF WE COULD --

DR. MILLAN: SO AS IT RELATES TO THE SPREADSHEET, WHAT WAS DONE IS, ON THE LEFT SIDE OF THE SPREADSHEET, YOU WILL SEE THE IND ENABLING, THAT'S THE SAME AS CLIN1. PHASE 1 OR 2 -- AND THEN REMAINING AWARDS ARE ALL CLIN2 AWARDS, BUT IT'S PHASE 1, 2, AND 3 ARE EACH OF THE PHASES OF THE TRIALS. AND SO THE GRAPH I'M SHOWING IS JUST A REPRESENTATION OF THE SMALLEST AWARD AMOUNT FOR THAT GIVEN CATEGORY AND THE LARGEST. SO IT'S THE RANGE OF AWARDS FOR A GIVEN CATEGORY. AND THEN THE AVERAGES FOR THOSE AWARD CATEGORIES IS A STAR, AND THE MEDIAN FOR ALL OF THOSE AWARDS IS IN THE HORIZONTAL LINE.

DR. DULIEGE: JUST ALSO CLARIFICATION BASED ON THE SPREADSHEET. YOU MENTIONED THE ICOC APPROVED AMOUNT FOR PHASE 3 TRIALS, AND THE PROPOSED CAP IS OBVIOUSLY LOWER THAN THAT. DOES IT MEAN THAT THIS MEASURE THAT YOU ARE RECOMMENDING, CAPPING AT $10 MILLION FOR PHASE 3, IS PROSPECTIVE, OR IS IT ALSO RETROSPECTIVE, WHICH I DOUBT IT WOULD BE, BUT

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TO CLARIFY?

DR. MILLAN: SO YOUR FIRST QUESTION, I'M GOING TO HAVE GABE THOMPSON, WHO'S OUR DIRECTOR OF GRANTS MANAGEMENT, COME RESPOND TO THAT. ACTUALLY YOU CAN RESPOND TO BOTH.

MR. THOMPSON: GABE THOMPSON. I'M DIRECTOR OF PORTFOLIO OPERATIONS. AND SO WHAT WE'VE HIGHLIGHTED IN THAT TABLE IS AWARDS THAT WE'VE ALREADY MADE THAT WOULD HAVE BEEN IMPACTED BY THESE NEW PROPOSED CAPS, BUT OUR PROPOSAL IS ONLY GOING FORWARD PROSPECTIVELY. BUT WE'VE HIGHLIGHTED THOSE AWARDS THAT WOULD HAVE BEEN EXCEEDING THAT.

DR. DULIEGE: JUST AS ANOTHER CLARIFICATION, I WOULD BE SURPRISED IF THERE ARE A LOT OF NOT-FOR-PROFIT REQUESTS FOR PHASE 3 FUNDING THAT COMES FROM NOT-FOR-PROFIT. AND, INDEED, THOSE THAT YOU HAVE HERE ARE, IF I'M CORRECT, ALL FOR-PROFIT, AS EXPECTED. DO YOU EXPECT ANY NOT-FOR-PROFITS DOING PHASE 3 TRIALS AND TRYING TO BE READY FOR COMMERCIALIZATION? I WOULD EXPECT VERY FEW, IF ANY, IN FACT, NONE.

DR. MILLAN: I THINK THAT'S OUR VIEW AS WELL.

CHAIRMAN THOMAS: DR. PRIETO.

DR. PRIETO: YES. QUESTION WITH REGARDS

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TO THE DIFFERENT CEILINGS OR AMOUNTS FOR PHASE 2 VERSUS PHASE 3. WOULDN'T THE PHASE 2 APPLICATIONS ALREADY HAVE AT LEAST SOME CLINICAL SAFETY DATA THAT WOULD ALSO PUT THEM IN A POSITION TO ATTRACT OUTSIDE FUNDING?

DR. MILLAN: YES. THEY WOULD HAVE SAFETY DATA BY PHASE 2. AND IT'S STILL -- ONE OF THE THINGS IS, EVEN WITH SAFETY DATA, IN TERMS OF CORPORATE OR PRIVATE INVESTORS, THEY REALLY STILL ARE LOOKING FOR EFFICACY DATA. THERE'S JUST THE FEEDBACK WE'VE GOTTEN FROM OUR PORTFOLIO PROGRAMS THAT HAVE BEEN GOING OUT FOR RAISES. WE CAN HAVE OUR DIRECTOR OF BUSINESS DEVELOPMENT COMMENT ON THAT, IF YOU WISH. BUT IN SOME CASES WE HAVE, THANKFULLY, BEEN ABLE TO ACHIEVE INVESTMENTS VERY EARLY, EVEN BEFORE CLINICAL DATA. SO THAT'S MORE OF THE EXCEPTION RATHER THAN THE RULE. BUT, IN GENERAL, SOME EFFICACY DATA AS WELL AS REALLY GETTING A GOOD READ. AS YOU KNOW, MANY TRIALS FAIL IN PHASE 2. SO THE APPETITE BY INDUSTRY INVESTORS, THERE IS MORE PULL, BUT IT'S NOT A VERY STRONG MAGNET YET.

CHAIRMAN THOMAS: MR. JUELSGAARD.

DR. JUELSGAARD: YES. DR. MILLAN, IN READING THE HANDOUT, AND I WANT TO GO TO PHASE 3 IN

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PARTICULAR, THE PROPOSED LIMITS THAT ARE UP THERE ARE $10 MILLION IN PHASE 3; AND YET IF I READ DOWN TO THE FIFTH LINE, BRAINSTORM, AND TO THE RIGHT HAND UNDER PROPOSED CAPS, IT SAYS 15 MILLION INSTEAD OF 10 MILLION. WHAT'S THE EXPLANATION FOR THE 15 IN PHASE 3 GIVEN THE NUMBER UP THERE?

DR. MILLAN: THE NUMBER THAT WE HAVE HERE IS THE PROPOSED CAPS GOING FORWARD. THE AWARDS THAT YOU WILL SEE IN THIS SUMMARY ARE THE CURRENT AWARDS WITH OUR CAP OF UP TO $20 MILLION FOR ALL PHASES.

DR. JUELSGAARD: I UNDERSTAND. DOES THE PROPOSED CAP, IT'S THE VERY FAR RIGHT COLUMN, OUT OF ALL THE NUMBERS, ALL THE 10S, THERE'S A 15, AND IT JUST STRIKES ME AS --

MR. THOMPSON: YOU ARE CORRECT. UNDER THE PROPOSED CAP, THE BRAINSTORM WOULD BE CAPPED AT 10 MILLION, NOT 15. THAT'S AN ERROR.

DR. JUELSGAARD: GOT IT. THANKS.

CHAIRMAN THOMAS: DR. LUBIN.

DR. LUBIN: FIRST OF ALL, THAT WAS A SUPERB PRESENTATION. WHAT DO YOU SEE AS THE DOWNSIDE OF DOING THIS? I MEAN YOU PRESENTED MOSTLY THE UPSIDE, WHICH WE ALL UNDERSTAND. WHAT DO YOU SEE AS THE POTENTIAL DOWNSIDE?

DR. MILLAN: THE POTENTIAL DOWNSIDE IS

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THAT WE MAY NOT BE BRINGING IN AS MANY PHASE 3 TRIALS BECAUSE, ESPECIALLY FROM THOSE THAT WOULD NEED TO COME IN FROM OUTSIDE CALIFORNIA, FOR INSTANCE, BECAUSE AT A CERTAIN POINT THEY HAVE TO MAKE THAT THEIR OWN CALCULATION AND BASED ON THEIR CORPORATE STRATEGY OF WHEN IT'S WORTH IT. SO IF IT'S A LOWER AMOUNT IN TERMS OF POTENTIAL NONDILUTED FUNDING, THAT'S A POTENTIAL RISK. HOWEVER, I'D LIKE TO EMPHASIZE THIS IS NONDILUTED FUNDING. SO WE HAVE FOUND THAT EVEN COMPANIES THAT HAVE A PRETTY SOLID FUNDING SOURCE FEEL THAT THIS IS STILL ENABLING AND ATTRACTIVE TO THEM AS A SOURCE OF NONDILUTED FUNDING.

DR. LUBIN: THANK YOU.

CHAIRMAN THOMAS: DR. STEWARD, DID YOU HAVE YOUR HAND UP AGAIN?

DR. STEWARD: I THINK YOU COVERED IT. THANK YOU.

CHAIRMAN THOMAS: DR. DULIEGE.

DR. DULIEGE: DO YOU WANT TO DISCUSS THIS NOW OR IS THAT ANOTHER TOPIC?

DR. MILLAN: SO THERE IS -- I'M GOING TO HAVE A COUPLE OF MORE SLIDES THAT ARE RELATED TO THIS TOPIC.

THE NEXT SLIDE IS ACTUALLY IF YOU WERE TO

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APPROVE THIS CLIN AWARD CAP, WHAT IT COULD LOOK LIKE IN TERMS OF WHAT WE COULD FUND. IF YOU DON'T APPROVE THE AWARD CAP REDUCTION, WE WOULD NOT BE ABLE TO ACTUALLY PLAN ON EVEN BRINGING TO YOU A PROPOSED BUDGET FOR DISC, TRAN, AND THE EDUCATION AWARDS THAT IS SHOWN HERE ON THIS SLIDE, THE PROPOSED SLATE OF PROGRAMS THAT WE WISH TO OFFER IN 2018.

SO I'LL JUST GO THROUGH THIS BRIEFLY. THE PROPOSED RESEARCH BUDGET ALLOCATION AND THE LONG-RANGE ARE TAKING INTO ACCOUNT 2018 AND 19 WITH THE REMAINING RESEARCH BUDGET.

FOR 2018 WE'RE ASKING THE BOARD TO APPROVE A TOTAL OF $130 MILLION TO FUND THE CLINICAL PROGRAMS. WE BELIEVE THAT, WITH THE REDUCED AWARD CAP, WOULD ALLOW US TO BRING IN 12 ADDITIONAL TRIALS IN 2018 AND FOUR CLIN1S, WHICH ARE THE IND-STAGE PROGRAMS. WE WOULD ALSO ASK THE BOARD FOR $30 MILLION TO FUND AT LEAST SIX TRAN PROGRAMS, 10 MILLION FOR SEVEN TO EIGHT DISC PROGRAMS, AND 750,000 FOR EDUCATION CONFERENCE AWARDS FOR ALPHA CLINICS, SPARK, AND BRIDGES PROGRAMS, AND ALL OF OUR PROGRAMS SO THAT THE KNOWLEDGE SHARING AND THE OUTPUT OF THOSE PROGRAMS COULD BE OPTIMIZED.

SO WITH THE NEXT SLIDE, IT'S KIND OF AN

OVERVIEW SLIDE. THIS BUDGET SCENARIO AND OUR PROPOSAL TO THIS BOARD IS CONSISTENT WITH THE FIVE-YEAR STRATEGIC GOALS IS PRESERVE THE FULL COMPLEMENT OF RESEARCH PROGRAMS, DISCOVERY, TRANSLATION, AND CLINICAL. IT PRESERVES CIRM'S ACCELERATION BY DESIGN OPERATION, AND THAT WOULD KEEP THE FUEL GOING THROUGH THAT ENGINE THAT YOU SAW IN TERMS OF ACTIVITIES, AWARDS, MANAGEMENT, AND DOING THAT EFFICIENTLY.

AND AS A SEPARATE TOPIC, WHICH WE WILL NOT BRING FORMALLY FOR ANY ACTION TODAY, THE ADMINISTRATION BUDGET SCENARIOS WHICH WE PRESENTED TO THE JOINT TRANSITION AND SCIENCE SUBCOMMITTEES IN NOVEMBER AND WILL BE PRESENTING FORMALLY TO THE BOARD IN MARCH FOR THE '18-'19 BUDGET. THE ADMINISTRATION BUDGET WOULD BE ABLE TO SUPPORT THE PERSONNEL REQUIRED FOR THE RESEARCH BUDGET PLAN.

SO THAT BRINGS ME, THEN, DR. DULIEGE, TO THE REQUESTED ACTION. THE CIRM TEAM REQUESTS THAT THE ICOC APPROVE THE PROPOSED REDUCTION IN MAXIMUM FUNDING LEVEL FOR CLIN AWARDS ACCORDING TO THE BELOW SCHEME. AND THOSE ARE THE NUMBERS THAT YOU SAW IN THE PREVIOUS CHART.

CHAIRMAN THOMAS: THANK YOU, DR. MILLAN. BEFORE WE ENTERTAIN A MOTION TO THAT EFFECT, ARE

THERE ANY QUESTIONS OR COMMENTS FROM MEMBERS OF THE BOARD ON THE PHONE ABOUT THE PRESENTATION? HEARING NONE, DR. DULIEGE HAS ANOTHER QUESTION.

DR. DULIEGE: JUST, MARIA, WOULD THERE BE A RATIONALE TO HAVE A DIFFERENT CAP BETWEEN NONPROFIT AND FOR-PROFIT FOR PHASE 2 WITH A SLIGHTLY HIGHER CAP FOR NONPROFIT, VERY MUCH AS WE HAD FOR CLIN AND CLIN1? JUST THINKING THAT BY THEN, THE FOR-PROFIT ORGANIZATIONS ALREADY HAVE TO HAVE A VISION ABOUT HOW THEY'RE GOING TO DO IT ALL THE WAY TO COMMERCIALIZATION, WHICH NONPROFITS MAY NOT ALREADY HAVE.

DR. MILLAN: THAT IS A CONSIDERATION. CERTAINLY THAT IS ANOTHER AREA WHERE POTENTIALLY WE CAN RECOVER MORE FOR THE RESEARCH BUDGET. IT'S NOT ONE WE'VE BROUGHT UP BECAUSE THE RISK, BY PHASE 2 WE'RE ASKING BOTH THE NONPROFITS AND FOR-PROFITS TO RISK SHARE WITH US WITH EACH BRINGING IN 40 PERCENT CO-FUNDING. ESSENTIALLY BY LOWERING THE AMOUNT, WHAT WE'RE ASKING THE FOR-PROFITS TO DO IS TO EVEN BRING IN MORE. AND THAT'S A CONSIDERATION. I GUESS THAT COULD BE LOOKED AT. WE HAVEN'T CONSIDERED THAT AT THIS TIME BECAUSE WE BELIEVE THAT THIS IS SOMETHING THAT'S STILL -- THIS PHASE IS STILL A PHASE AT CIRM IS ESSENTIAL TO EVEN A PHASE 2.

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DR. STEWARD: I ACTUALLY HAVE TWO QUESTIONS. I'M NOT CALLING FOR PUBLIC COMMENT BECAUSE IT'S NOT THE APPROPRIATE TIME. I'M JUST CURIOUS. IS THERE GOING TO BE PUBLIC COMMENT ABOUT THIS? CAN SOMEBODY BACK THERE RAISE YOUR HAND? ANY ON THE PHONE? I'M JUST CURIOUS. THANK YOU.

AND THE SECOND -- THERE IS ONE. AS I SAY, I'M NOT ASKING THAT WE DO IT NOW, BUT I JUST WANTED TO KNOW HOW MUCH THERE WAS GOING TO BE BECAUSE I THINK IT'S GOING TO BE USEFUL PERHAPS TO LEAVE SOME TIME FOR THE BOARD DISCUSSION IN RESPONSE TO ANY PUBLIC COMMENT THAT WE MIGHT HAVE.

CHAIRMAN THOMAS: I THINK THE PROCEDURE HERE, DR. STEWARD, WOULD BE WE WOULD ENTERTAIN A MOTION TO ADOPT THE RECOMMENDATION, AT WHICH POINT IN THE PROCESS OF DEBATING THAT, THERE WOULD BE PUBLIC COMMENT ON THAT.

DR. STEWARD: THANK YOU.

AND THE SECOND QUESTION, AND THIS IS ONE THAT I THINK WE TALKED ABOUT IN THE SUBCOMMITTEE MEETING, I JUST HAVE TO SAY YOU'VE DONE A GREAT JOB OF PRESENTING THIS. I KNOW THAT YOU AND THE REST OF THE TEAM HAVE DONE JUST A SUPERB AMOUNT OF WORK. THERE'S A LOT OF CHANGE HERE. I'M JUST CURIOUS. AT WHAT POINT WILL YOU SORT OF LOOK AT AND SAY, OOPS,

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THIS ISN'T WORKING OR THIS IS WORKING? JUST A QUESTION ABOUT THAT.

DR. MILLAN: THANK YOU. IF THIS CHANGE LED TO A SIGNIFICANT DROP IN OUR ABILITY TO BRING IN HIGH QUALITY PROGRAMS TO OUR PORTFOLIO, WE WOULD COME BACK TO THE BOARD AND REPORT ON THAT AND BRING AN ALTERNATE PROPOSAL BACK TO YOU.

DR. STEWARD: DO YOU HAVE A TIME FRAME FOR WHEN YOU MIGHT TAKE A LOOK AT THAT?

DR. MILLAN: WE LOOK AT IT CONTINUALLY. AS WE HAVE QUARTERLY MEETINGS, WE HAVE QUARTERLY IN-PERSON MEETINGS, I THINK THERE'S AN OPPORTUNITY TO BRING IT UP AT ANY TIME. EVEN IF WE NEEDED TO CALL A SPECIAL MEETING, I THINK THAT THAT'S SOMETHING THAT WE COULD DO; BUT AT LEAST EVERY QUARTER, WE HAVE AN IN-PERSON MEETING, AND PRIOR TO THAT THERE WOULD BE SUBCOMMITTEE MEETINGS.

DR. STEWARD: SO I HAVE THE MICROPHONE. MAY I MAKE A MOTION TO APPROVE THE PROPOSAL AS IT'S THERE?

CHAIRMAN THOMAS: THANK YOU, DR. STEWARD. IT'S BEEN MOVED. IS THERE A SECOND?

DR. LUBIN: SECOND.

CHAIRMAN THOMAS: SECONDED BY DR. LUBIN. DISCUSSION BY MEMBERS OF THE BOARD EITHER HERE OR ON

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THE PHONE? DR. DULIEGE.

DR. DULIEGE: JUST WANTED, PER MY PREVIOUS COMMENTS, I WOULD BE HAPPY TO APPROVE THIS MOTION AS SUCH, BUT I DON'T KNOW IF THERE'S A POSSIBILITY TO GIVE LEEWAY FOR CIRM TO CAP, FURTHER DISCUSSION ON THAT TO CAP THE PHASE 2 FOR-PROFIT A LITTLE BIT LOWER FOR THE REASONS I MENTIONED. I DON'T THINK WE SHOULD DISCUSS IT NOW, BUT IF CIRM HAS THE LEEWAY TO APPLY THAT, THAT WOULD BE GREAT.

CHAIRMAN THOMAS: I THINK WE CAN ACCOMMODATE THAT. I THINK ALSO, DR. DULIEGE, THAT'S PART OF THE ANALYSIS THAT DR. MILLAN AND THE TEAM WILL DO ON AN ONGOING BASIS HERE TO SEE HOW THIS PLAYS OUT.

AND, DR. STEWARD, IN REFERENCE TO YOUR QUESTION ABOUT SORT OF WHEN DO WE KNOW THINGS AREN'T WORKING, I THINK THAT THIS WHOLE IDEA CAME ABOUT AS A RESULT OF JUST THAT ANALYSIS. AND I WOULDN'T SAY THAT THINGS WEREN'T WORKING. I WOULD SAY THINGS WERE WORKING SO WELL THAT WE HAD TO MAKE AN ADJUSTMENT TO RECOMMEND THE CAPS TO ALLOW US TO CONTINUE WITH THE PROGRAMS. BUT I THINK THAT DR. MILLAN AND THE TEAM WILL UNDERGO ONGOING AND CONTINUED ANALYSIS AND REPORT BACK TO US ON THAT.

DR. HIGGINS.

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DR. HIGGINS: WOULD YOUR SUPPORT FOR THIS PROPOSAL DIFFER IF THE OTHER STRATEGY WE HAVE BEEN TALKING ABOUT TODAY OF HOW TO SORT OF BRIDGE FUNDING TO THE FUTURE GOES ONE WAY OR THE OTHER? IT'S A QUESTION FOR YOU, MARIA.

DR. MILLAN: SO THE BUDGET SCENARIOS I PRESENTED TODAY ARE INDEPENDENT OF ANY OTHER EXTERNAL SOURCES OF FUNDING WITH WHAT IS ALREADY ALLOCATED UNDER PROP 71.

CHAIRMAN THOMAS: OTHER QUESTIONS, COMMENTS, FOR MEMBERS OF THE BOARD ON THE MOTION? HEARING NONE, DO WE HAVE PUBLIC COMMENT? PLEASE GIVE YOUR NAME.

DR. CHIU: ARLENE CHIU, CITY OF HOPE. I HAVE A NUMBER OF QUESTIONS. THE FIRST IS HOW MUCH DOES AN NIH-FUNDED CLINICAL TRIAL ON AVERAGE COST? AND I KNOW THIS IS A VERY BROAD QUESTION BECAUSE THERE ARE SO MANY INSTITUTES FUNDING DIFFERENT KINDS. SO I'M HOPING -- THAT'S MY FIRST QUESTION.

MR. THOMPSON: SO I DON'T HAVE THAT EXACT ANSWER. I'VE ATTEMPTED TO LOOK UP WHAT NIH FUNDS. I'M PRETTY SURE THEY FUND LESS THAN WHAT WE HAVE HISTORICALLY FUNDED. IT MAY BE CLOSER TO WHAT IS BEING PROPOSED HERE, BUT NIH WILL OFTEN FUND TRIALS IN THE CONTEXT OF LARGER INFRASTRUCTURE AWARDS. AND

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SO IT'S HARD FOR ME TO TEASE OUT. WE FUND DIRECTLY THE CLINICAL TRIAL; WHEREAS, THEY'RE FUNDING A LARGER KIND OF INFRASTRUCTURE AROUND CLINICAL TRIALS.

DR. CHIU: THANK YOU. IN MY EXPERIENCE, YOUR CURRENT CAPS ARE EXCEEDINGLY GENEROUS. AND JUST BASED ON AN UNDERSTANDING OF HOW GRANTEES WRITE PROPOSALS, THEY WILL ASK UP TO THE LIMIT. MANY OF THEM WOULD. AND SO I'M ASSUMING THAT THESE VERY WISE PROPOSALS WILL NOT NECESSARILY LIMIT -- CREATE THE DIRE CIRCUMSTANCES THAT SOME MIGHT HAVE BEEN ANTICIPATING. I'M NOT SAYING NOME, BUT I'M JUST SAYING MIGHT NOT.

MY SECOND QUESTION IS REGARDING THE CONSEQUENCES OF THIS, OF NOT DOING THIS ACTION. AND IS IT CORRECT THAT IF YOU MAINTAIN YOUR CURRENT CAPS OF 20 MILLION, THAT YOU WILL NOT BE ABLE TO HAVE ANY BUDGET MONIES LEFT FOR ANY TRAN OR DISCOVERY PROPOSALS IN 2018 AND 2019? I'D LIKE A CLARIFICATION ON THAT.

DR. MILLAN: BASED ON THE CURRENT AWARD SIZES AND THE CURRENT PERFORMANCE, THAT'S CORRECT, AND WE CERTAINLY COULDN'T COUNT ON IT. WE COULDN'T PREDICT. IN TERMS OF FORECASTING, WE NEED TO USE WHAT WE HAVE IN OUR EXPERIENCE AND OUR DATA. YES,

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THAT'S CORRECT.

DR. CHIU: THANK YOU VERY MUCH.

CHAIRMAN THOMAS: ADDITIONAL PUBLIC COMMENT? THANK YOU.

DR. NICHOLAS: I'M CORY NICHOLAS, CO-FOUNDER AND CFO OF NEURONA THERAPEUTICS IN SOUTH SAN FRANCISCO. I APOLOGIZE. I'M LOSING MY VOICE ON A DAY WHEN I REALLY WANT TO USE IT.

I WANT TO APPLAUD CIRM'S PROGRESS, AND I ALSO WANT TO SAY THAT I APPRECIATE THE CHALLENGE THAT CIRM AND ICOC HAVE IN STRATEGICALLY DISTRIBUTING THESE DOLLARS TO ACCOMPLISH YOUR MISSION AND ACCELERATE EFFECTIVE THERAPIES FOR PATIENTS IN NEED. I WANT TO JUST SAY THAT I SUPPORT THE CAP; BUT, IN FACT, I RECOMMEND A MORE AGGRESSIVE CAP ON THESE CLINICAL PROGRAMS BECAUSE IT'S REALLY A DOUBLE-EDGED SWORD. AND IT'S THESE EARLIER STAGE DISCOVERY AND TRAN PROGRAMS THAT WILL SUFFER AS A RESULT OF INCREASED CLINICAL SPENDING.

AND I WANT TO SAY THAT YOU HAVE A NUMBER OF REALLY PROMISING AND STRONG EARLIER DISCOVERY AND TRAN PROGRAMS THAT ARE IN STRIKING DISTANCE OF THE CLINIC, IN FACT. MANY OF THESE PROGRAMS HAVE BEEN FUNDED BY CIRM SINCE THEIR INCEPTION, AND THEY JUST NEED A LITTLE MORE SUPPORT TO GET OVER THE HUMP TO

GET INTO LATER PRECLINICAL AND CLINICAL DEVELOPMENT.

SO THIS IS WHY IT'S SO TROUBLING TO SEE THE DISCOVERY AND TRAN BUDGETARY CUTS PROPOSED IN 2018 AND 2019. IN FACT, THE DISCOVERY BUDGET, EVEN WITH THIS CAP, IS GOING TO BE REDUCED FIVEFOLD, AND THE TRANSLATIONAL BUDGET IS GOING TO BE CUT IN HALF.

IN CONTRAST, EVEN WITH THIS CAP, THE CLINICAL BUDGET IS STILL GOING TO BE ONE AND A HALF TIMES HIGHER THAN IT WAS IN 2016. SO I REALLY STRONGLY URGE YOU TO RECONSIDER YOUR PROPOSED BUDGETARY ALLOCATIONS AND CONSIDER A REVISED, MORE STRINGENT CLINICAL CAP AND MAINTAIN A HEALTHY BALANCE IN YOUR PORTFOLIO. CONTINUE TO BUILD UPON YOUR INVESTMENT IN YOUR EARLIER STAGE PROGRAMS THAT YOU'VE BEEN FOSTERING FOR SO LONG.

IT'S REALLY GOING TO BE THESE EARLY PROGRAMS AS THEY MATURE THAT ARE GOING TO BE DRIVING CIRM'S CLINICAL PORTFOLIO IN THE YEARS TO COME. THANK YOU.

CHAIRMAN THOMAS: ADDITIONAL PUBLIC COMMENT?

DR. LORING: I'M JEANNE LORING. I'M FROM THE SCRIPPS RESEARCH INSTITUTE, AND I'M ALSO LOSING MY VOICE.

I'M NOT SPEAKING ON BEHALF OF MYSELF HERE.

I'M SPEAKING ON BEHALF OF SOMEBODY WHO COULDN'T COME BECAUSE SHE'S VERY, VERY ILL. I THINK MOST OF YOU HAVE MET JENNIFER RAUB. SHE'S A FIERCE ADVOCATE FOR STEM CELL THERAPY FOR PARKINSON'S DISEASE. AND SHE WANTED TO SPEAK TODAY BECAUSE OF OUR CHALLENGES IN OBTAINING CIRM FUNDING FOR PARKINSON'S DISEASE. I'LL BE REALLY BRIEF.

JENNIFER IS THE PRESIDENT OF SUMMIT4STEMCELL FOUNDATION. IT'S A GRASS ROOTS FOUNDATION OF PATIENT ADVOCATES THAT HAVE PARKINSON'S DISEASE. JENNIFER HAS PARKINSON'S DISEASE, AND SHE WANTS TO BE TREATED WITH A STEM CELL THERAPY THAT WOULD REVERSE THE DOWNWARD SLIDE THAT IS INEVITABLE FOR PEOPLE WITH PARKINSON'S DISEASE.

THERE ARE FOUR GROUPS WORLDWIDE WHO ARE DEVELOPING THERAPIES, NEURON REPLACEMENT THERAPIES, FOR PARKINSON'S DISEASE. THEY PLAN TO HAVE -- ALL OF THEM PLAN TO HAVE THEIR THERAPIES IN THE CLINIC BY 2018 OR 2019. THE FOUR GROUPS ARE COORDINATING THEIR EFFORTS IN A PARTNERSHIP CALLED G FORCE, AN INTERNATIONAL PARTNERSHIP ORGANIZATION, TO COORDINATE EFFORTS.

IN NEW YORK THE NEW YORK STEM CELL EQUIVALENT OF CIRM HAS INVESTED 20 MILLION IN HUMAN

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EMBRYONIC STEM CELL-DERIVED NEURONS. THE SECOND PROJECT IS A PARTNERSHIP BETWEEN UK AND SWEDEN WHO RECEIVED MORE THAN $20 MILLION FROM THE EUROPEAN UNION. AND FINALLY, THE THIRD IS THE JAPANESE GOVERNMENT THAT HAS INVESTED MORE THAN $20 MILLION IN AN EQUIVALENT PROJECT.

THE FOURTH PROJECT IN G FORCE IS OUR PROJECT. WE ARE DEVELOPING A PATIENT-SPECIFIC DOPAMINE NEURON REPLACEMENT THERAPY. JENNIFER, THROUGH SUMMIT, HAS RAISED MORE THAN $3 MILLION THAT HAS SUPPORTED US SO FAR. IN ORDER TO REACH CLINICAL TRIAL BY 2019, WE NEED FURTHER FUNDING. CIRM HAS GRANTED US $2.4 MILLION, BUT WE APPLIED TWICE FOR A TRANSLATIONAL GRANT AND HAVE BEEN TURNED DOWN.

CIRM HAS SPENT LESS THAN 1.6 PERCENT OF ITS BUDGET FOR PARKINSON'S DISEASE. THIS DISEASE WAS HIGHLIGHTED BY MICHAEL J. FOX AND JOAN SAMUELSON IN THE EFFORT TO GET PROP 71 FUNDED. SINCE OUR GRANTS HAVE BEEN REJECTED, WE ARE LOSING MONEY ELSEWHERE. SUMMIT IS RAISING MORE MONEY. THERE'S ANOTHER NONPROFIT THAT'S RAISING MONEY FOR US, AND THERE ARE SEVERAL DONORS WHO ARE WILLING TO PUT MONEY INTO THIS PROJECT.

WHAT DISAPPOINTS ME IS THAT CIRM DOESN'T WANT TO CONTINUE THIS PROJECT. I THINK THIS IS

SOMETHING THAT WE NEED TO DISCUSS, THAT CERTAIN DISEASES, FOR SOME REASON, HAVE BEEN OVERLOOKED DURING CIRM'S FUNDING. WE WILL BE IN THE CLINIC IN 2019 WITH OR WITHOUT CIRM. TIME'S UP.

CHAIRMAN THOMAS: IS THERE ADDITIONAL PUBLIC COMMENT ON THIS MOTION? HEARING NONE, MARIA, WILL YOU CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: YES.

MS. BONNEVILLE: LARS BERGLUND.

DR. BERGLUND: AYE.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: DEBORAH DEAS. JACK DIXON.

DR. DIXON: YES.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: HOWARD FEDEROFF. JUDY GASSON.

DR. GASSON: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

DR. JUELSGAARD: YES.

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MS. BONNEVILLE: SHERRY LANSING. BERT LUBIN.

DR. LUBIN: YES.

MS. BONNEVILLE: LINDA MALKAS.

DR. MALKAS: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: AYE.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OSWALD STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

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CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: KRISTINA VUORI.
DR. VUORI: YES.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: THE MOTION CARRIES.
CHAIRMAN THOMAS: THANK YOU, DR. MILLAN. WILL YOU CONTINUE WITH YOUR PRESENTATION PLEASE.
DR. MILLAN: THANK YOU, CHAIRMAN THOMAS.
SO THE NEXT REQUESTED ACTION FROM THIS BOARD, BASED ON YOUR APPROVAL OF THE AWARD CAP REDUCTION FOR CLINICAL AWARDS, IS THE FOLLOWING. THE CIRM TEAMS REQUESTS THAT YOU APPROVE THE 2018 RESEARCH BUDGET ALLOCATION OF $130 MILLION FOR CLINICAL PROGRAMS, $30 MILLION FOR TRANSLATIONAL, $10 MILLION FOR DISCOVERY AWARDS.
CHAIRMAN THOMAS: DO I HEAR A MOTION TO THAT EFFECT?
DR. HIGGINS: SO MOVED.
CHAIRMAN THOMAS: MOVED BY DR. HIGGINS.
DR. DULIEGE: SECOND.
CHAIRMAN THOMAS: SECONDED BY DR. DULIEGE. QUESTIONS OR COMMENTS BY MEMBERS OF THE BOARD EITHER

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HERE OR ON THE PHONE? DO WE HAVE PUBLIC COMMENT? WE DO HAVE PUBLIC COMMENT FOR THOSE ON THE PHONE. WE ARE GETTING THE MICROPHONE TO OUR SPEAKER.

DR. CHAZENBALK: GOOD MORNING, EVERYBODY. I WANT TO THANK --

CHAIRMAN THOMAS: CAN YOU PLEASE GIVE YOUR NAME AND AFFILIATION?

DR. CHAZENBALK: MY NAME IS GREGORIO CHAZENBALK. I'M A PH.D, BASIC SCIENTIST, AND PROFESSOR OF OB/GYN AT UCLA. NOW I WANT TO THANK CIRM TO GIVE ME THE OPPORTUNITY TO SPEAK HERE TODAY. AND BRIEFLY I WANT TO TALK ABOUT A DISCOVER THAT WE DID IN OUR LAB, AND WE HAVE APPLIED FOR A GRANT THAT WAS NOT GRANTED.

AS EVERYBODY KNOWS, (UNINTELLIGIBLE) THE STEM CELLS ARE (UNINTELLIGIBLE) AND THEY INDUCE PLURIPOTENCY.

DR. STEWARD: POINT OF ORDER. I BELIEVE THAT WE'RE DISCUSSING THE MOTIONS HERE AND THAT DISCUSSIONS OF INDIVIDUAL GRANTS SHOULD BE RESERVED UNTIL THE END OF THE MEETING.

CHAIRMAN THOMAS: YES, THAT'S CORRECT. THIS IS NOT GERMANE TO THE MOTION.

DR. CHAZENBALK: I WAS INFORMED THAT MY TALK WOULD BE IN THE MORNING. I'M COMING FROM LOS

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ANGELES, AND I HAVE A --

CHAIRMAN THOMAS: IT WILL BE. WE JUST HAVEN'T REACHED THE APPROPRIATE AGENDA TOPIC YET. THANK YOU. WE'LL GET BACK TO YOU AT THE APPROPRIATE TIME. THANK YOU.

OTHER COMMENTS BY MEMBERS OF THE PUBLIC? HEARING NONE, THIS, I BELIEVE, REQUIRES ONLY A VOICE VOTE EXCEPT FOR THOSE ON THE PHONE. FOR THOSE IN THE ROOM, ALL IN FAVOR OF THIS MOTION PLEASE SAY AYE. OPPOSED? ABSTENTIONS? MARIA, PLEASE CALL THE ROLL FOR THOSE ON THE PHONE.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: YES.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: JACK DIXON.

DR. DIXON: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

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MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: YES.

MS. BONNEVILLE: THE MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU VERY MUCH, DR. MILLAN. ANY CONCLUDING?

DR. MILLAN: I'D LIKE TO THANK THE BOARD FOR CONSIDERING THESE PROPOSALS AND FOR YOUR SUPPORT OF WHAT WE DO AT CIRM. THANK YOU.

CHAIRMAN THOMAS: THANK YOU VERY MUCH.

WE'RE GOING TO GO -- CHANGE ONE AGENDA ITEM OUT OF ORDER HERE AND PROCEED TO ITEM NO. 8, WHICH IS THE CONSIDERATION OF APPLICATIONS SUBMITTED FOR DISC2, WHICH ARE OUR AWARDS. I'LL TURN THIS OVER NOW TO SUPERVISOR SHEEHY.

SUPERVISOR SHEEHY: THANK YOU, CHAIRMAN THOMAS.

SO I THINK THE WAY WE TYPICALLY PROCEED IS WE TAKE A MOTION TO MOVE ANY APPLICATION OUT OF TIER I INTO TIER II. IS THERE A MOTION?

CHAIRMAN THOMAS: MR. SUPERVISOR, I THINK DR. SAMBRANO HAD A --

SUPERVISOR SHEEHY: I FORGOT HIS PRESENTATION. I'M SORRY. THINGS ARE A LITTLE RATTLED HERE UNFORTUNATELY.

DR. SAMBRANO: WE ARE PULLING UP THE

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PRESENTATION RIGHT NOW. THANK YOU VERY MUCH. THIS IS GIL SAMBRANO. I'M THE VICE PRESIDENT FOR PORTFOLIO DEVELOPMENT AND REVIEW AT CIRM.

I JUST WANTED TO GIVE YOU AN OVERVIEW OF THE DISCOVERY QUEST PROGRAM FOR WHICH WE ARE BRINGING RECOMMENDATIONS FROM THE GRANTS WORKING GROUP. AS YOU KNOW, WE HAVE AND CONTINUE TO HAVE FUNDING OPPORTUNITIES ACROSS DIFFERENT PILLARS AS WAS DISCUSSED. THE CORE OF THOSE ARE THE DISCOVERY, TRANSLATION, AND CLINICAL PROGRAMS. THE QUEST PROGRAM FITS SQUARELY WITHIN DISCOVERY, AND WE CONSIDER IT TO BE THE WORKHORSE OF THE DISCOVERY PROGRAM IN ORDER TO BRING SINGLE PRODUCT CANDIDATES FORWARD THAT WILL BE READY FOR TRANSLATION. AND THE OBJECTIVE OF THIS PROGRAM IS, IN FACT, TO DO THAT, TO LOOK FOR PROMISING NEW STEM CELL-BASED TECHNOLOGIES THAT CAN ADVANCE TO TRANSLATION WITHIN TWO YEARS IN ORDER TO ULTIMATELY IMPROVE PATIENT CARE.

SO WHAT QUALIFIES FOR QUEST? SO THE QUEST PROGRAM HELPS SUPPORT PROGRAMS THAT ARE LAUNCHING A PRODUCT CANDIDATE THAT'S EITHER A THERAPEUTIC, A DIAGNOSTIC, A MEDICAL DEVICE, OR A TOOL. IT IS ONE OF OUR BROADEST PROGRAMS, AND THIS NEXT SLIDE JUST HIGHLIGHTS THAT. IN TERMS OF THERAPY, WE LOOK AT

ANYTHING FROM A STEM PROGENITOR CELL THERAPY, ALSO REPROGRAMMED CELL THERAPIES, SMALL MOLECULES OR BIOLOGICS THAT ACT ON A STEM CELL OR CANCER STEM CELL. FOR DEVICES, DIAGNOSTIC, OR TOOLS, THOSE THAT IN SOME WAY WOULD USE STEM CELL OR PROGENITOR CELLS OR THAT ADDRESS A CRITICAL BOTTLENECK IN THE STEM CELL THERAPY FIELD.

THE REVIEW CRITERIA THAT WE UTILIZE FOR THE GWG TO REVIEW AND EVALUATE THESE APPLICATIONS ARE SHOWN IN THIS SLIDE. AND THERE ARE FOUR BASIC QUESTIONS THAT WE PROVIDE TO THEM AS GUIDANCE. THE FIRST IS DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT? THAT IS, WHAT IS THE VALUE THAT THE PROJECT BRINGS, AND HOW WELL DOES IT ALIGN WITH THE MISSION OF THE PROGRAM? IS THE RATIONALE SOUND? THAT IS, DOES IT MAKE SENSE? DOES THE APPLICANT BRING SUFFICIENT SUPPORTING DATA IN ORDER TO SUPPORT THE WORK THAT IS PROPOSED? IS THE PROJECT WELL-PLANNED AND DESIGNED? AND IS THE PROJECT FEASIBLE, INCLUDING HAVING AN APPROPRIATE TEAM AND ALL THE RESOURCES THAT ARE NEEDED TO CARRY OUT THE PROJECT AND ALSO ACCOMPLISH IT WITHIN THE TWO-YEAR TIMELINE?

THE SCORING SYSTEM THAT IS UTILIZED IS ONE TO A HUNDRED. UNDER THIS PROGRAM, ANYTHING THAT'S

GIVEN A SCORE OF A 85 TO A HUNDRED MEANS THAT IT'S RECOMMENDED FOR FUNDING IF FUNDS ARE AVAILABLE. A SCORE OF 1 TO 84 MEANS THAT IT'S NOT RECOMMENDED FOR FUNDING. WE USE THE MEDIAN OF SCORES BY THE SCIENTIFIC MEMBERS OF THE GWG IN ORDER TO ASCERTAIN THE FINAL SCORE FOR EACH APPLICATION.

SO THIS TABLE SUMMARIZES THE RECOMMENDATIONS FROM THE GWG. THERE WERE 43 APPLICATIONS THAT WERE REVIEWED BY THE GWG FOR THE QUEST PROGRAM. THERE WERE 11 APPLICATIONS THAT RECEIVED A SCORE BETWEEN 85 AND A HUNDRED THAT ARE RECOMMENDED. AND THE TOTAL FUNDING REQUESTS TO COVER THOSE 11 APPLICATIONS IS ABOUT $21 MILLION. THE FUNDS THAT ARE AVAILABLE UNDER THE PROGRAM FOR THIS YEAR IS ABOUT 25.8 MILLION, SO WE ARE WELL WITHIN THE ALLOWABLE BUDGET FOR THE PROGRAM TO FUND ALL 11 PROGRAMS.

MR. CHAIRMAN, AT THIS POINT I HAVE AN OVERVIEW OF RECOMMENDED APPLICATIONS THAT WE CAN GO THROUGH. OR IF MEMBERS HAVE LOOKED AT IT, WE CAN SKIP OVER THAT, BUT I WILL PROCEED AS YOU WISH.

SUPERVISOR SHEEHY: I THINK, IN GENERAL, I HOPE PEOPLE LOOKED AT THEIR MATERIALS. AND IT SEEMS LIKE WE HAVE A FAIRLY PACKED AGENDA TODAY. IT MIGHT MORE SENSE, AS WE DO OUR REGULAR ORDER, IF PEOPLE

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WANTED QUESTIONS ABOUT A SPECIFIC APPLICATION.

MR. TORRES: MR. CHAIRMAN, IS IT APPROPRIATE NOW TO MOVE AN APPLICATION INTO THE FUNDING SEGMENT?

SUPERVISOR SHEEHY: I THINK THE FIRST THING THAT WE DO -- THE USUAL ORDER IS THAT WE WOULD FIRST TAKE MOTIONS TO MOVE APPLICATIONS FROM TIER I TO TIER II. AND THEN WE MAKE A MOTION TO MOVE APPLICATIONS FROM TIER II TO TIER I. AND THEN WHAT WE DO AFTER THAT IS TAKE A MOTION A FINAL MOTION ON ALL THE APPLICATIONS.

IS THERE A MOTION TO MOVE ANY APPLICATION FROM TIER I TO TIER II?

DR. STEWARD: COULD I JUST ASK FOR ONE CLARIFICATION BEFORE WE START THAT PROCESS? AND THIS IS ACTUALLY A REQUEST OF SCOTT TOCHER, TO EXPLAIN THE SITUATION IN TERMS OF THE FUNDING CAP AND THAT THERE'S A POTENTIAL FOR SOME MOVES TO PUT US OVER THE CAP, WHICH PUTS SOME OF US IN CONFLICT. SO IF YOU CAN JUST EXPLAIN THE NATURE OF THAT AND WHY THAT LIMITS DISCUSSION BY SOME OF US FOR SOME MOTIONS GOING FORWARD.

MR. TOCHER: SURE. THE RECOMMENDATION FROM THE GRANTS WORKING GROUP, THE APPLICATIONS IN THE GREEN TIER I DO NOT QUITE MEET THE BALANCE

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REMAINING ON THE FUNDS FOR THIS YEAR FOR THIS PROGRAM. HOWEVER, THERE ARE NUMEROUS APPLICATIONS THAT MAY BE THE SUBJECT OF MOTIONS TO MOVE UP TO TIER I FROM TIER II THAT COULD EXCEED THE CAP THAT IS AVAILABLE. THAT WOULD THEN ENTAIL THE APPLICATION REVIEW SUBCOMMITTEE TO THEN TAKE VOTES TO MOVE APPLICATIONS BACK OUT OF TIER I TO ENSURE THAT THE FUNDING DOES NOT EXCEED THE CAP. THEREFORE, WE ADVISE MEMBERS WITH INTEREST IN ANY APPLICATION IN EITHER TIER I OR TIER II TO ABSTAIN FROM DISCUSSION OR PARTICIPATION IN THE VOTE OF ANY OF THESE UNTIL WE HAVE AN OMNIBUS MOTION TO FUND OR NOT FUND THE REMAINING APPLICATIONS.

SUPERVISOR SHEEHY: THANK YOU, SCOTT. DO WE HAVE A MOTION TO MOVE ANY APPLICATIONS FROM TIER I TO TIER II? NOT HEARING A MOTION, DO WE HAVE A MOTION TO MOVE ANYTHING FROM TIER II TO TIER I?

MR. TORRES: YES, MR. CHAIRMAN. I WOULD LIKE TO MOVE TO TIER II -- TO TIER I THE SPINAL CORD INJURY NEURAL STEM CELL SPINAL CORD INJURY FOR A TOTAL OF 2.1 MILLION WITH THE UNDERSTANDING THAT IF WE EXCEED, THEN WE HAVE TO MAKE ANOTHER DECISION AT THAT POINT. THIS IS A PROJECT FUNDED AT UC SAN DIEGO.

SUPERVISOR SHEEHY: IS THERE A SECOND TO

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THAT MOTION?

DR. HIGGINS: I'LL SECOND THAT.

SUPERVISOR SHEEHY: WE HAVE A MOTION AND A SECOND. ANY BOARD DISCUSSION ON THE MOTION?

DR. PRIETO: ACTUALLY I HAVE A QUESTION FOR DR. SAMBRANO. FOR APPLICATIONS THAT ARE NOT FUNDED, WHAT IS THE OPPORTUNITY FOR APPLICANTS TO COME BACK?

DR. SAMBRANO: WE WANT TO MAKE AS MUCH AS POSSIBLE ALL OUR PROGRAMS TO HAVE RECURRING AND PREDICTABLE OPPORTUNITIES. SO THE NEXT OPPORTUNITY FOR THIS ONE, WE ANTICIPATE THE DEADLINE WILL BE IN MARCH OF NEXT YEAR.

SUPERVISOR SHEEHY: ADDITIONAL QUESTIONS, COMMENTS FROM THE BOARD?

DR. JUELSGAARD: SO IN LOOKING AT THE REPORT THAT STANDS BEHIND THE CHART THAT'S SHOWN UP HERE, THERE'S A ONE-, TWO-, THREE-PAGE REPORT. BUT UNDER CRITERIA, THE ONE THAT I NOTED, SO THERE ARE FOUR CRITERIA THAT ARE USED, THE THIRD ONE, AND THIS IS NOW THE GWG'S CRITERIA, IS THIS PROPOSAL WELL-PLANNED AND DESIGNED? AND THE THREE OUTCOMES THAT WERE INDICATED ARE POSITIVE, INCLUDES NEGATIVE INFLUENCE, OR NEUTRAL INFLUENCE. SO WITH RESPECT TO IS THE PROPOSAL WELL-PLANNED AND DESIGNED, THERE

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WERE FIVE THAT GAVE IT A POSITIVE INFLUENCE, FIVE THAT GAVE IT A NEGATIVE INFLUENCE, AND FOUR THAT FELT IT WAS NEUTRALLY INFLUENCED.

SO THE CONCERN, AT LEAST ON MY PART, AND WE'RE DEALING WITH THE GWG NOW, AN ORGANIZATION THAT WE'VE ASKED TO VALIDATE THE SCIENTIFIC CREDIBILITY OF THESE PROJECTS, WHEN THEY TALK ABOUT THE DESIGN OF A STUDY, THE PLANNING AND DESIGN, AND DON'T GIVE MORE RINGING ENDORSEMENTS THAN WHAT WE SEE ON THIS, IT'S NOT TO SUGGEST THAT THIS ISN'T A WORTHWHILE AREA TO PURSUE, BUT THE QUESTION IS IS THIS REALLY THE RIGHT PLAN FOR PURSUING IT. AND SO I WOULD SIMPLY MAKE THAT OBSERVATION AS SOMETHING TO AT LEAST THINK ABOUT WITH RESPECT TO A VOTE ON THIS ISSUE.

SUPERVISOR SHEEHY: ANY ADDITIONAL COMMENTS?

CHAIRMAN THOMAS: DR. DULIEGE HAS HER ARM UP, MR. SUPERVISOR.

SUPERVISOR SHEEHY: WOULD YOU MIND TO COMMENT -- DR. SAMBRANO, WOULD YOU MIND TO COMMENT ON THE DIFFERENCES BETWEEN THE GWG ON ONE HAND AND THE LETTER THAT WE ALL READ THAT WAS SENT BY DR. TUSZYNSKI, THE PI? AND COULD YOU HELP US WITH THIS?

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DR. SAMBRANO: I WILL DO MY BEST TO HELP YOU. SO, IN GENERAL, LET ME JUST PREFACE THIS BY SAYING IN TERMS OF GWG COMMENTS, I CAN SPEAK TO WHAT THEY GENERALLY BELIEVED OR WHAT THEY THOUGHT OF AN APPLICATION. IT'S DIFFICULT, BECAUSE THEY'RE NOT HERE, TO UNDERSTAND TO WHAT EXTENT WE CAN INFLUENCE THOSE CONCERNS. SO OUR PROCESS, IN GENERAL, WE ASK APPLICANTS TO RESUBMIT, ADDRESS CONCERNS SO THAT THE GWG CAN DETERMINE WHETHER THEY'VE ADEQUATELY ADDRESSED THOSE CONCERNS. AND THAT HAS HAPPENED GENERALLY. SO YOU WILL SEE THAT SOME OF THESE APPLICATION RESUBMISSIONS, WHERE THE APPLICANT HAS MADE A RESUBMISSION, THE GWG HAS ACKNOWLEDGED IT, AND THEY HAVE SHOWN AN IMPROVEMENT.

SO IN TERMS OF THIS PARTICULAR APPLICATION, I THINK OVERALL THIS DID NOT HAVE ANY MAJOR ISSUES. A LOT OF THE CONCERNS WERE RELATIVELY MINOR, NO FATAL FLAWS. REVIEWERS WERE CERTAINLY CONCERNED ABOUT SOME OF THE PRELIMINARY DATA IN TERMS OF WHETHER IT SHOWED THE DEVELOPMENT OF A RELAY IN SPINAL CORD INJURY. BUT IN TERMS OF A PROJECT THAT CAN MOVE FORWARD INTO TRANSLATION, IN TERMS OF A PROJECT THAT HAS A GOOD TEAM, I THINK REVIEWERS FELT COMFORTABLE THAT IT MET THOSE CRITERIA. I THINK WHAT THEY REALLY WERE CONCERNED

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ABOUT WAS THE PRELIMINARY DATA THAT DEMONSTRATED THAT THE MECHANISM BY WHICH THIS MAY WORK MAY NOT BE FULLY SUPPORTED.

WE HAVE LOOKED AT SOME OF THIS INFORMATION, THE DATA; AND, AS MENTIONED, THIS HAS NO MAJOR CONCERNS. IT DOES OFFER SOME PROGRAMMATIC VALUE IN TERMS OF OFFERING A DIFFERENT APPROACH TO SPINAL CORD INJURY COMPARED TO OTHER PROJECTS THAT WE ARE FUNDING. AND SO IT'S SOMETHING THAT COULD ADD VALUE TO OUR PORTFOLIO.

SUPERVISOR SHEEHY: OTHER QUESTIONS OR COMMENTS? DR. DULIEGE, DO YOU HAVE OTHER QUESTIONS YOU WANT TO ASK?

DR. DULIEGE: I WANT TO BE VERY CAREFUL HERE BECAUSE I THINK, IN GENERAL, WE ARE TRYING NOT TO OVERRIDE THE DECISION OR THE RECOMMENDATION MADE BY THE GWG GROUP. HERE WHAT I HEAR IS THERE IS NO MAJOR FLAWS. IN FACT, IT WOULD ADD TO OUR PIPELINE. AND SO I'LL LOVE IF I CAN HAVE A LITTLE BIT OF AN EXPLANATION AS TO WHY THE SCORE WAS NOT HIGHER, AND IT IS THE CASE THERE WERE NO VERY LOW SCORE. THEY WERE ALL PRETTY CLOSE TO THE MEDIAN BEING 80 AND THE LOWER. SO THIS IS THE ONE THAT WE COULD POTENTIALLY OVERRIDE, AND WE WANT TO BE CAREFUL BEFORE DOING THAT.

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SUPERVISOR SHEEHY: PUBLIC COMMENT?

MR. REED: THIS IS DON REED. AS YOU ALL KNOW, I'VE BEEN INVOLVED IN SPINAL CORD INJURY RESEARCH FUNDING FOR 23 YEARS, AND I'VE KNOWN THE APPLICANT FOR THAT LONG. AND HE'S AN UNDERSALESMAN OF WHAT HE DOES. HE IS A SUPERB SCIENTIST.

NOW, A COUPLE OF THINGS. IT'S REALLY HARD FOR ME SOMETIMES TO LOOK AT A PHOTOGRAPH OF NERVE REGENERATION AND REALIZE WHAT I'M LOOKING AT. IT'S USUALLY LIKE YOU'VE GOT THE NOTCH IN THE SPINE AND YOU'VE GOT A LITTLE FUZZ AND YOU'RE SUPPOSED TO INTERPRET THAT SOMEHOW.

WITH THE PHOTOGRAPHS OF HIS, YOU SEE THE NERVE LEAPING ACROSS THE BARRIER. THIS IS SOMETHING SUPERB.

ALSO, IT'S IMPORTANT THAT, ALTHOUGH HE DOESN'T TALK ABOUT THE CHRONIC ASPECTS, CHRONIC IS HUGE. PEOPLE ARE ONLY PARALYZED IN THE ACUTE PHASE FOR A COUPLE WEEKS. WHEN YOU'RE IN A CHRONIC, YOU'RE A LONG TIME. AND EVERYBODY IN THE WORLD THAT'S PARALYZED PRETTY MUCH IS CHRONIC. HE'S DONE THIS CHRONIC WORK WITH PRIMATES. NO ONE ELSE HAS DONE THAT. PRIMATES, IT'S MONKEYS. THIS IS A BIG STEP FORWARD.

THIS IS PROBABLY THE CULMINATION OF HIS

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LIFE'S WORK. IT'S ALSO BRINGING TOGETHER FIVE OTHER UNIVERSITIES' TOP PEOPLE. IT'S A SUPERB PROJECT. I RECOMMEND IT STRONGLY.

CHAIRMAN THOMAS: ADDITIONAL PUBLIC COMMENT HERE, JEFF.

SUPERVISOR SHEEHY: IN THE PUBLIC COMMENT.

CHAIRMAN THOMAS: WE HAVE A COUPLE MORE SPEAKERS.

MR. KLEIN: THIS IS BOB KLEIN. I'M SPEAKING AS AN INDIVIDUAL. I WOULD JUST LIKE TO STRESS THAT CONSTITUTIONALLY IT IS VERY IMPORTANT THAT THE BOARD EXERCISE INDEPENDENT JUDGMENT WHEN THERE'S A MERITORIOUS REASON TO EXERCISE THAT JUDGMENT. IT IS VERY IMPORTANT THAT CONSTITUTIONALLY THE BOARD NOT COMPLETELY IDENTIFY AND ADOPT ALL POSITIONS OF THE PEER REVIEW COMMITTEE AS IT CREATES ISSUES THAT WERE PROPERLY ADDRESSED IN THE CONSTITUTIONAL LITIGATION.

SO WITHOUT COMMENTING ON THE CASE BEFORE YOU, WHICH YOU ALL HAVE TO EVALUATE, I THINK IT IS VERY IMPORTANT THAT THE BOARD FEEL EMPOWERED AND UNDERSTAND THE NECESSITY, AS PART OF ITS ROLE CONSTITUTIONALLY WITHIN THE STATE, TO MAKE DECISIONS AT TIMES, PERHAPS BY EXCEPTION, BUT TO MAKE DECISIONS WHERE THE MERITS COMPEL A FINDING OF VALUE

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IN THE PORTFOLIO.

CHAIRMAN THOMAS: ANOTHER SPEAKER, MR. SUPERVISOR.

DR. TUSZYNSKI: GOOD MORNING. I'M MARK TUSZYNSKI. I'M THE LEAD INVESTIGATOR ON THE PROPOSED PROJECT. THANK YOU FOR THE OPPORTUNITY TO ADDRESS YOU.

SO I LEAD A CONSORTIUM OF INVESTIGATORS FROM FIVE UNIVERSITY OF CALIFORNIA CAMPUSES WORKING ON THIS PROGRAM. I'D LIKE TO FOCUS MY COMMENTS ON SORT OF PROGRAMMATIC ISSUES RELATED TO CIRM AND ITS FUTURE DIRECTION.

SO WE ARE A CONSORTIUM OF PEOPLE AT UCSD, UC IRVINE, UCLA, UCSF, AND UC DAVIS. WE HAVE STUDIED NEURAL STEM CELLS FROM A VERY DISTINCT APPROACH, AS SOMEBODY MENTIONED, FROM THE EXISTING PROGRAM SUPPORTED BY CIRM. THE EXISTING PROGRAMS THAT ARE IN CLINICAL USE THROUGH ASTERIAS TARGET THE RESTORATION OF FUNCTION TO CONNECTIONS AFTER A SPINAL CORD INJURY THAT ARE ACTUALLY SPARED AFTER THE INJURY. AND THAT'S A HIGHLY MERITORIOUS PROJECT. IT'S GREAT THAT THAT'S MOVING FORWARD, BUT OUR APPROACH IS VERY FUNDAMENTALLY DIFFERENT.

WE TRY TO FILL IN THE INJURY SITE ITSELF WITH NEURAL STEM CELLS THAT, IN TURN, SEND OUT NEW

CONNECTIONS THAT ARE MEANT TO FORM RELAYS. AND ON THE QUESTION OF WHETHER WE HAVE SHOWN RELAYS, WE THINK WE HAVE. THOSE FINDINGS WERE PUBLISHED IN THE JOURNAL *CELL* IN 2012 BY MY COLLEAGUE DR. LU, THAT SHOWED ELECTRICAL CONDUCTION ACROSS THE RELAY AND FUNCTIONAL IMPROVEMENT AFTER SEVERE SPINAL CORD INJURY, WHICH IS A MODEL SO DIFFICULT TO STUDY, THAT MOST PEOPLE IN SPINAL CORD INJURY DON'T EVEN APPROACH IT. YET WE SAW FUNCTIONAL IMPROVEMENT AND RELAYS ACROSS THAT MODEL.

SO FROM A PROGRAMMATIC BASIS, WE HAVE DEVELOPED THIS TECHNOLOGY. WE STAND ON THE VERGE OF TRANSLATION. OUR GOAL IS TRANSLATION. AND WE'RE IN THE VALLEY OF DEATH THAT YOU MENTIONED EARLIER WHERE WE HAVE TO DO THESE STUDIES TO GENERATE THE LEAD CANDIDATE CELL TYPE TO GO INTO CLINICAL TRIALS. WE ARE POISED TO DO THAT WITH A DISTINCT APPROACH FROM PROGRAMS ALREADY FUNDED BY CIRM.

WE ARE A CONSORTIUM OF INVESTIGATORS. WE HAVE TRANSFERRED OUR TECHNOLOGY TO NONHUMAN PRIMATES TO DEVELOP THE MODELS AND TOOLS. I THINK WE'RE THE ONLY SPINAL CORD INJURY GROUP THAT HAVE MOVED THIS TECHNOLOGY TO A NONHUMAN PRIMATE MODEL, AND WE ARE POISED TO MOVE FORWARD.

IT'S VERY HARD TO GET FUNDING AT THIS

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LEVEL OF THE VALLEY OF DEATH, PRECISELY THE PROGRAMMATIC MISSION OF CIRM. I DON'T KNOW THAT FOR THE NEXT STAGE OF WORK REQUIRED WE WOULD BE SUCCESSFUL IN ANOTHER ARENA. AND I'M HAPPY TO SAY THAT IN THE LAST FIVE YEARS, WE'VE PUBLISHED THE RESULTS OF OUR WORK IN SEVEN LEAD JOURNALS IN THE FIELD OF SCIENCE AND MEDICINE, INCLUDING TWO PAPERS IN *NATURE MEDICINE*, A PAPER IN THE JOURNAL *CELL*, TWO PAPERS IN *SCIENCE TRANSLATIONAL MEDICINE*, *JOURNAL OF CLINICAL INVESTIGATION*. THESE ARE ALL TRANSLATIONAL JOURNALS THAT HIGHLIGHT THE TRANSLATIONAL FOCUS OF OUR WORK.

SO I ENCOURAGE YOU TO CONSIDER THIS AS BEING A SECOND SHOT ON GOAL FOR THE PROBLEM OF SPINAL CORD INJURY, AN AREA OF GREAT UNMET MEDICAL NEED, AND WITH POTENTIAL IN CHRONIC INJURY TOO. THANK YOU VERY MUCH.

SUPERVISOR SHEEHY: ADDITIONAL PUBLIC COMMENT?

DR. LU: GOOD MORNING. MY NAME IS PAUL LU. I'M FROM UNIVERSITY OF CALIFORNIA SAN DIEGO. AS EVERYBODY CAN SEE, I'M IN A WHEELCHAIR BECAUSE I HAD A TERRIBLE CAR ACCIDENT CAUSED SPINAL CORD INJURY 20 YEARS AGO. IT'S UNFORTUNATE FOR ME, BUT IT'S FORTUNATE I HAVE A CHANCE TO PARTICIPATE ON

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SPINAL CORD INJURY RESEARCH WITH DR. MARK TUSZYNSKI. I WANT TO EMPHASIZE I MYSELF, WITH SUPPORT OF OUR TEAM, DEVELOPED A NEW METHOD TO SUPPORT STEM CELL SURVIVE MATURATION IN THE SEVERE SPINAL CORD INJURY. AND WITH THIS SUPPORT, WHEN A NERVE CELL MATURE, IT BECOMES NERVE, AND WE SEE GREAT CONNECTIVITY OF THE GRAFT NERVE WITH THE HOST. AND DEAL FROM AND ATOMIC AND ELECTRIC WE HAVE THIS EVIDENCE, AND WE CONSTANT HAVE THIS RESULT IN A DIFFERENT KIND OF CELLS. SO FOR THIS GRANT, IT'S VERY CRITICAL. AND WITH TRANSLATION EARLY FINDING TO THE HUMAN NEURAL STEM CELL, THAT WILL GOING TO CLINIC.

ON THE OTHER HAND, I'M SPINAL CORD PATIENT MYSELF AND RESEARCH AND HAVE DOUBLE POSITION. I WAS CONSTANT CONTACT BY OTHER SPINAL CORD INJURY PATIENT. AND THE SPINAL CORD INJURY PATIENT HAVE GREAT HOPE FOR THE STEM CELL TO CURE THE SPINAL CORD INJURY. AND I ATTEND A LOT OF MEETINGS, AND WE THANK CALIFORNIA FOR THIS SPECIAL CIRM ADDITIONAL STEM CELL FUNDING TO SUPPORT OUR STUDY. AND I'M PRETTY SURE WITH THIS SUPPORT WE WILL PUSH THIS PROJECT TO THE CLINIC AND TRANSLATION. AND EVERYBODY IN THE SPINAL CORD COMMUNITY HOPE TO GET THIS FUNDING TO SPEED UP THE STEM CELL TREATMENT FOR SPINAL CORD INJURY. THANK YOU.

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SUPERVISOR SHEEHY: ANY ADDITIONAL PUBLIC COMMENT?

CHAIRMAN THOMAS: YES.

DR. CHIU: I'M ARLENE CHIU FROM THE CITY OF HOPE. AND I HAVE TO DRAW BACK MORE THAN 20 YEARS WHEN I WAS AT THE NIH AND I WAS PROGRAM DIRECTOR FOR THE SPINAL CORD INJURY PROGRAM. AND I HAVE SEEN A LOT OF SPINAL CORD INJURY RESULTS PROPOSALS. I HAVE TO SAY THAT THE ASTERIAS PROJECT THAT YOU HAVE SUPPORTED SO GREATLY DEPENDS ON THE EXISTENCE OF ALREADY SURVIVING CONNECTIONS BETWEEN THE BRAIN AND WITHIN THE SPINAL CORD WHERE REMYELINATION WILL PROMOTE THE RESIDUAL ACTIVITY. THIS IS SOMETHING VERY DIFFERENT THAT YOU HAVEN'T SUPPORTED YET. AND OVER THE YEARS, PEOPLE HAVE SUGGESTED THIS MECHANISM, BUT HAVE SCANT DATA TO SHOW RELAYS AND RECONNECTING. AND THEN ESPECIALLY FOR CHRONIC SPINAL CORD INJURY, THIS IS A VERY HIGH BAR.

TO HAVE BROUGHT THIS PROJECT BY DR. TUSZYNSKI TO THIS POINT IN THE STATE OF CALIFORNIA IS NO MEAN FEAT. AND SO I WOULD HOPE THAT YOU WOULD GIVE IT A SECOND LOOK AND TRY TO SUPPORT SUCH A LONG-TERM AND CONSISTENT EFFORT IN BRINGING RESULTS TO A TERRIBLE CHRONIC SITUATION IN PATIENTS.

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CHAIRMAN THOMAS: I'M ASKING FOR MR. SUPERVISOR HERE ANY MORE PUBLIC COMMENT? YES, THERE IS. ONLY FOR THIS APPLICATION. I SEE NO MORE, MR. SUPERVISOR, ALTHOUGH WE DO HAVE SOME COMMENTS BY MEMBERS OF THE BOARD.

SUPERVISOR SHEEHY: YES.

DR. PRIETO: I WANTED TO RESPOND TO STEVE JUELSGAARD'S COMMENTS. AND WHILE I VERY MUCH RESPECT THE EXPERTISE AND THE WORK THAT'S DONE FOR US BY THE MEMBERS OF THE GWG, AND I SERVE ON THE GWG, I WOULD LIKE TO REITERATE WHAT BOB KLEIN SAID TO US AND THE CONTROVERSY THAT CAME UP IN A VERY MAJOR WAY IN THE EARLY DAYS OF CIRM, WHICH IS THAT THE BOARD HAS A RESPONSIBILITY TO EXERCISE OUR INDEPENDENT JUDGMENT AND TO WEIGH THE ISSUES LIKE PROGRAMMATIC CONCERNS AND OUR OWN ASSESSMENT OF OUR RISK TOLERANCE WHEN WE WEIGH APPLICATIONS LIKE THIS. SO WE ARE REQUIRED TO MAKE THOSE INDEPENDENT JUDGMENTS AND NOT RUBBER STAMP THE OPINION OF THE GWG.

THAT SAID, I THINK THAT WITH CIRM 2.0 WE ARE GIVING APPLICANTS AN OPPORTUNITY TO COME BACK TO US IN A RELATIVELY SHORT PERIOD OF TIME, BUT I THINK IT'S IMPORTANT THAT WE CONSIDER APPLICATIONS LIKE THAT THAT ARE ON THE BORDER OF OUR FUNDING LINE.

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THANK YOU.

CHAIRMAN THOMAS: DR. DULIEGE HAS A COMMENT.

DR. DULIEGE: JUST ACTUALLY MORE QUESTION. THIS FIRST REQUEST HAS COME ON THIS PROPOSAL, BUT THERE ARE MANY OTHER REQUESTS THAT MAY COME UP IN A MINUTE OR SO. ARE WE CAPPED TO MAXIMUM BUDGET, OR CAN WE EVALUATE THESE REQUESTS INDEPENDENT OF EACH OTHER?

SUPERVISOR SHEEHY: WE'RE CAPPED.

DR. SAMBRANO: WE ARE CAPPED AT ABOUT 25.8 MILLION. THE REASON THESE ARE NOW BLUE IS TO SHOW, PRESUMABLY, IF THE BOARD APPROVES THE ELEVEN, WE WOULD BE AT 21 MILLION, AND THERE ARE 4.8 APPROXIMATELY AVAILABLE FOR ADDITIONAL FUNDING. THE BUDGET REQUESTED IS SHOWN NEXT TO EACH ONE. FROM THAT, YOU CAN CALCULATE MAYBE TWO OR THREE PROJECTS THAT, IF THEY WERE TO BE BROUGHT UP, COULD POSSIBLY BE FUNDED.

SUPERVISOR SHEEHY: I WOULD JUST SAY THAT WE SHOULD BE VERY CAREFUL ABOUT EXPANDING OUR UNIVERSE. THESE ROUNDS OCCUR OFTEN, AND I THINK IT'S OKAY TO DO A LITTLE BIT, BUT I DO THINK THAT IT IS CHALLENGING IF WE SUDDENLY START APPROVING A NUMBER OF THESE AND WE START HAVING TO REALLY GO --

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LET'S PUT IT LIKE THIS. IT WOULD BE VERY UNFORTUNATE IF WE HAD TO TAKE SOMETHING OUT OF THE FUNDABLE CATEGORY BECAUSE THEY'RE NOT HERE. AND THEY GOT GREAT SCORES AND WE EXCEEDED OUR BUDGET.

SO I DO WARN US TO BE CONSCIOUS OF THE FACT THAT WE HAVE A LIMITED BUDGET, WE HAVE A CAP THAT WE CANNOT EXCEED.

DR. DULIEGE: SO IN THIS CONTEXT, WOULD IT BE FAIR TO NOTE, IF THERE'S GOING TO BE OTHER MOTIONS TODAY, SO THAT WE CAN LOOK AT THE RELATIVE MERIT OF THE OTHER AND NOT END UP SAYING NO TO ONE BECAUSE THERE'S NO MORE BUDGET?

SUPERVISOR SHEEHY: THAT'S NOT IN OUR PROCESS, AND I WORRY ABOUT HOW THAT WOULD WORK.

WE HAVE A MOTION ON THE FLOOR.

MR. TORRES: THAT IS CORRECT.

SUPERVISOR SHEEHY: AND WE SHOULD VOTE THAT MOTION. AND IF THERE ARE ADDITIONAL MOTIONS, WE'LL HAVE TO CONSIDER IN THOSE MOTIONS WHETHER OR NOT WE HAVE THE FUNDING TO FUND THOSE APPLICATIONS OR IF WE WANT TO TAKE ANOTHER APPLICATION OUT OF THE FUNDABLE CATEGORY. ALL THE MATERIALS RELATED TO THE GRANTS HAVE BEEN AVAILABLE TO BOARD MEMBERS FOR ENOUGH TIME FOR THEM TO STUDY IT AND COME TO CONCLUSIONS ABOUT WHICH ONES THEY WANT TO MOVE OR

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NOT MOVE.

MR. TORRES: MR. CHAIRMAN, I JUST WANTED TO ADD AS WELL THAT IF THESE GRANTS, ANY OF THESE GRANTS, DO NOT MEET THEIR MILESTONES, THEY COULD BE CUT OFF FROM FUNDING, CORRECT?

SUPERVISOR SHEEHY: YES, BUT THAT DOESN'T ADDRESS OUR CHALLENGE.

MR. TORRES: I KNOW. BUT WE'VE ALSO REACHED A POINT TO WHERE SOME GRANTS DID NOT GET APPROVAL BECAUSE WE DIDN'T HAVE ENOUGH MONEY EITHER. AND THAT WAS IN A PREVIOUS ROUND THAT WE HAD. SO IT IS NOT -- IT IS VERY CHALLENGING. THERE'S NO QUESTION ABOUT THAT. AND I THINK AT THIS POINT WE'RE DEALING WITH IT AD SERIATIM, AND BOARD MEMBERS NEED TO FIGURE OUT WHETHER THEY WANT TO CONSIDER OTHER MOTIONS DOWN THE ROAD AND THEN MAKE A DECISION UPON THIS MOTION, BUT I WOULD MOVE THAT WE HAVE A ROLL CALL VOTE.

CHAIRMAN THOMAS: MR. SUPERVISOR, I JUST HAVE ONE LAST QUESTION BEFORE WE DO THAT, IF YOU WOULD, MR. SENATOR.

DR. SAMBRANO, NORMALLY IF THERE ARE APPLICATIONS THAT THE GWG VIEWED AS HAVING SOME MATERIAL FLAWS OF ONE SORT OR ANOTHER, THAT WOULD BE REPORTED BACK TO THE PI WITH AN IDEA THAT THEY COULD

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RECTIFY THOSE POTENTIALLY IN SUBSEQUENT APPLICATIONS. YOU'VE NOTED WITH RESPECT TO THIS APPLICATION THAT THERE WERE NO MAJOR FLAWS IDENTIFIED. WERE THERE ANY SUGGESTIONS RELAYED IN THE REPORTS BACK TO THE PI HERE THAT WOULD GIVE THEM AN IDEA OF HOW TO AUGMENT THEIR PROPOSAL WERE THEY TO COME BACK THE NEXT ROUND?

DR. SAMBRANO: WE ARE ALSO HAPPY TO WORK WITH ALL THE APPLICANTS, SO EVEN THOSE THAT ARE IN THE TOP TIER. THOSE HAVE ALSO NOTATIONS ABOUT CONCERNS THAT GWG MEMBERS HAVE. SO IN THE PROCESS OF SETTING UP AN AWARD, WE UTILIZE THAT TO TRY TO MAKE IMPROVEMENTS AS WE MOVE FORWARD WITH THEM.

CHAIRMAN THOMAS: THAT SEEMS TO BE ALL THE QUESTIONS AT THIS END, MR. SUPERVISOR.

SUPERVISOR SHEEHY: WELL, THEN I'M GOING TO ASK MARIA TO CALL THE ROLL. BEFORE I DO, I JUST WANT TO MAKE ONE ADDITIONAL COMMENT AS THE CHAIR. THIS IS WHERE WE ARE. WE HAVE A FINITE AMOUNT OF MONEY. AND I DON'T KNOW THAT WE'VE REALLY COME TO TERMS WITH THIS, BUT WE HAVE TO BE VERY, VERY CONSCIOUS OF WHAT WE SPEND. SO ANYWAY, PLEASE CALL THE ROLL, MARIA.

MS. BONNEVILLE: ANNEMARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.
DR. HIGGINS: YES.
MS. BONNEVILLE: STEPHEN JUELSGAARD.
DR. JUELSGAARD: ABSTAIN.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: ABSTAIN.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: ABSTAIN.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.

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MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: IS THERE A MOTION TO TAKE ANY OTHER APPLICATION FROM TIER II AND MOVE IT INTO TIER I?

IS THERE AN OMNIBUS MOTION TO FUND ALL THE APPLICATIONS IN TIER I AND NOT --

MR. TORRES: SO MOVED.

SUPERVISOR SHEEHY: -- FUND ANY APPLICATIONS IN TIER II? THERE'S TWO PARTS TO IT. IT'S BEEN MOVED BY SENATOR TORRES. DO WE HAVE A SECOND?

CHAIRMAN THOMAS: WE HAVE A QUESTION HERE, MR. SUPERVISOR.

DR. MARTIN: I HAVE A TECHNICAL QUESTION. THAT IS ON THE APPLICATION 10748, WHICH IS AN HIV PROPOSAL. THE TECHNICAL QUESTION IS IN THE GWG WAS THERE AN HIV EXPERT IN THAT COMMITTEE THAT PARTICIPATED IN THIS REVIEW? I PRESUME THERE WAS, BUT WAS THERE?

SUPERVISOR SHEEHY: YES.

DR. MARTIN: I'M SORRY. MY QUESTION ABOUT THE PROPOSAL AND THE CONCERNS OF THIS IS THAT THE MODEL THAT'S BEING USED IS NOT RELEVANT TO THE PROBLEM OF HIV INFECTION. IT'S A CHALLENGING --

CHAIRMAN THOMAS: I WOULD DISAGREE.

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DR. MARTIN: BUT THE PROBLEM IS THE CRYPTIC INFECTION OR THE LATENT INFECTION AND NOT A CHALLENGE MODEL, AND SO --

SUPERVISOR SHEEHY: PRIMATE MODEL BY HANS (INAUDIBLE) AT UNIVERSITY OF WASHINGTON GENERALLY CONSIDERED THE CELL THERAPY TO BE THE BEST. HE QUOTES THE BEST INVESTIGATOR, AND THIS IS THE BEST MODEL IN USE.

DR. MARTIN: I AGREE. CAR-T'S ARE VERY VALID TO THIS. I'M JUST CONCERNED ABOUT THE MODEL, THE CHALLENGE MODEL. THANK YOU FOR ANSWERING THE QUESTION.

CHAIRMAN THOMAS: DIANE HAS A COMMENT, MR. SUPERVISOR.

MS. WINOKUR: MAYBE AT THIS POINT IT WOULD BE HELPFUL TO JUST BRIEFLY DESCRIBE FOR THE PEOPLE WHO ARE HERE OBSERVING HOW THIS GRANT WORKING GROUP REALLY WORKS, LIKE THE MAKEUP OF IT AND THE DISCUSSION AND THE HOURS THAT ARE SPENT ON EACH PROPOSAL.

DR. SAMBRANO: CERTAINLY I CAN JUST GIVE YOU VERY BRIEFLY AN OVERVIEW OF THE PROCESS. SO ALL APPLICATIONS GO THROUGH A PANEL OF 15 SCIENTISTS AND 7 PATIENT ADVOCATES WHO COMPOSE THE GWG. IN ADDITION TO THAT, DEPENDING ON THE EXPERTISE THAT'S

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REQUIRED AS WE LOOK AT THE PORTFOLIO OF APPLICATIONS THAT COME, WE ALSO RECRUIT WHAT WE CALL SPECIALISTS TO ADD ADDITIONAL EXPERTISE TO THE PANEL. ALL OF THE APPLICATIONS ARE ASSIGNED TO A MINIMUM OF THREE DIFFERENT SCIENTISTS IN THAT GROUP, AND THEN WE BRING THEM ALTOGETHER TO HAVE AN IN-DEPTH DISCUSSION OF EACH APPLICATION BEFORE THE ENTIRE PANEL SO EVERYBODY CAN CONTRIBUTE TO THAT DISCUSSION AND UNDERSTAND WHAT THE STRENGTHS, CONCERNS WERE BEFORE THEY SCORE ON THAT APPLICATION.

DOES THAT SUFFICIENTLY SUMMARIZE?

CHAIRMAN THOMAS: NO OTHER COMMENTS FROM MEMBERS OF THE BOARD HERE IN THE ROOM.

SUPERVISOR SHEEHY: DO WE HAVE A SECOND FOR THE MOTION?

MR. TOCHER: NOT YET, JEFF.

MS. WINOKUR: I SECOND.

SUPERVISOR SHEEHY: SO ANY FURTHER BOARD DISCUSSION? PUBLIC COMMENT?

DR. SANTO: I'M ELLEN SANTO, PROFESSOR OF PEDIATRICS AND CELL BIOLOGY AT UC DAVIS SCHOOL OF MEDICINE. I'M ALSO REPRESENTING MY COLLEAGUE AND COLLABORATOR SIMON CHERRY, WHO'S A PROFESSOR OF BIOMEDICAL ENGINEERING AND RADIOLOGY.

WE THANK THE BOARD FOR THE OPPORTUNITY TO

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REQUEST THE CONSIDERATION OF OUR QUEST APPLICATION 10599, TRANSLATIONAL IMAGING TOOL FOR HUMAN REGENERATIVE THERAPIES. THE GOAL OF THIS APPLICATION IS TO DEMONSTRATE A TRANSFORMATIVE NEW IMAGING APPLICATION SPECIFICALLY FOR POSITRON EMISSION TOMOGRAPHY OR PET FOR STEM CELL THERAPEUTICS. CIRM IS IN A UNIQUE POSITION TO SET THE STAGE FOR USE OF THIS NEW TECHNOLOGY AND WHY CALIFORNIA MAINTAINS THE LEADERSHIP POSITION.

WHILE PET HAS VERY HIGH SENSITIVITY AND CAN PROVIDE THREE-DIMENSIONAL IMAGES DEEP INSIDE THE HUMAN BODY, CURRENT PET SYSTEMS CAN ONLY EVALUATE A SMALL ANATOMICAL AREA AT A GIVEN TIME. IN FACT, TODAY'S PET SCANNERS ONLY CAPTURE LESS THAN 1 PERCENT OF THE SIGNAL BECAUSE THE MAJORITY OF THE BODY IS NOT INSIDE THE SCANNER AT A GIVEN MOMENT IN TIME. THE INNOVATIVE CONCEPT OF TOTAL BODY PET OR WHAT WE CALL EXPLORER WILL BE GROUNDBREAKING BY COLLECTING MORE THAN 40-FOLD SIGNAL AND SIGNIFICANTLY ADVANCING IMAGING FOR EVERY DISEASE THAT CIRM SUPPORTS ACROSS THE LIFE SPAN, INCLUDING FOR THE YOUNGEST PATIENTS IN NEED.

A SCALED VERSION OF EXPLORER IS CURRENTLY AVAILABLE FOR IMMEDIATE USE OF THE STUDIES PROPOSED, AND THE WORLD'S FIRST HUMAN SCANNER WILL BE

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OPERATIONAL AT THE UC DAVIS MEDICAL CENTER IN 2018. WITH THE HUMAN SCANNER NEAR COMPLETION, PROTOCOLS THAT HAVE BEEN FULLY OPTIMIZED AND VALIDATED AND NEEDED IN ORDER TO ENSURE TO NOT DELAY HUMAN APPLICATION.

THE EXPLORER TECHNOLOGY IS ABLE TO PERFORM TOTAL BODY STUDIES AT 140TH THE CURRENT RADIATION DOSE USED, ALLOWING SCANS AT FRACTIONS OF THE EXPOSURE INDIVIDUALS RECEIVE, FOR EXAMPLE, FOR A ROUND TRIP FROM SAN FRANCISCO TO LONDON. EXPLORER TECHNOLOGY CAN IMAGE WITH ENHANCED SENSITIVITY; IT CAN IMAGE FASTER BY IMAGING THE ENTIRE BODY AT ONCE. IT CAN IMAGE SAFELY WITH SIGNIFICANTLY REDUCED RADIATION DOSE, WHICH THIS ALLOWS NEW APPLICATIONS FOR PEDIATRICS AND IMAGE MORE OFTEN. THUS, CALIFORNIA AND CIRM WILL BE A LEADER IN DEVELOPING AND APPLYING THIS TECHNOLOGY FROM REGENERATIVE MEDICINE FROM PEDIATRICS TO GERIATRICS.

THE STUDIES PROPOSED IN THIS QUEST APPLICATION WILL INITIATE AND ESTABLISH A NEW FIELD OF TOTAL BODY PET IMAGING IN REGENERATIVE MEDICINE USING SOPHISTICATED SIMULATION TOOLS IN ORDER TO DEVELOP QUANTITATIVE METHODS FOR IN VIVO IMAGING. CELL DOSES WILL BE BASED ON THESE STUDIES AND CONDUCTED IN A HIGHLY RELEVANT PRIMATE MODEL WITH

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IMMENSE TRANSLATIONAL VALUE.

OUR APPLICATION RECEIVED AN 84, JUST MISSING THE CUTOFF BY ONE POINT. WE HOPE THAT THE BOARD WILL CONSIDER OUR APPLICATION, ALLOWING EXPLORER TO FILL A GAP AND CURRENT MEDICAL NEED BY PROVIDING A STEM CELL IMAGING TECHNOLOGY THAT WE BELIEVE WILL BE TRANSFORMATIVE AND SIGNIFICANTLY IMPROVE PATIENT CARE ACROSS THE LIFE SPAN. THANK YOU.

SUPERVISOR SHEEHY: NEXT.

DR. CHAZENBALK: IT'S MY TURN. I HOPE THAT THE 30 SECONDS I TALK BEFORE WILL NOT BE TAKEN. SO, AGAIN, MY NAME IS GREGORIO CHAZENBALK. I WORK AT UCLA IN STEM CELL. IN 2010 A NOVEL PUBLICATION CAME ABOUT REPORTING STEM CELLS WITHOUT TERATOGENESIS. MEANS THEY WILL NOT PRODUCE TERATOMAS IN CONTRAST TO STEM CELLS IN IPS. THESE CELLS CALLED NEW CELLS ARE HIGHLY RESISTANT TO SEVERAL TRAITS. THE NAME IS TYPE IMPORTANT, AND YOU ARE STRESSING STEM CELLS HAVE THE ABILITY TO DIFFERENTIATE TO ANY KIND OF CELLS WAS PUBLISHED BY DIFFERENT INVESTIGATORS. THERE ARE TEN GROUPS WORLDWIDE THAT DEMONSTRATED THESE CELLS, AND THESE CELLS ALSO HAVE (UNINTELLIGIBLE), WHICH MEANS THEY CAN GO ONLY TO THE INJURY AREA BECAUSE THEY HAVE A

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SPECIFIC RECEPTOR THAT RESPONDS TO MOLECULE USED BY ANY TISSUE INJURY.

SO THESE CELLS ALREADY ARE THE MOST THAT CAN REGENERATE TISSUE AND RESTORE FUNCTION IN MANY DISEASES, LIKE ISCHEMIC ULCER, FIBROSIS, KIDNEY DAMAGE, STROKE. PAPER PUBLISHED IN 2016 IN STEM CELL JOURNAL AND RECENTLY A PAPER IS COMING ABOUT EFFECT OF THESE CELLS TO REGENERATE IN ACUTE MYOCARDIAL INFARCTION. THE REGENERATION OF THE HEART AND (UNINTELLIGIBLE) HAVE BEEN DEMONSTRATED IN MICE, RATS, PIGS, AND THERE ARE ALREADY ONGOING CLINICAL TRIALS IN JAPAN SPONSORED BY MITSUBISHI AND UNIVERSITY OF TOHOKU AND TOKYO UNIVERSITY SHOWING ANOTHER PROMISING OF THESE CELLS.

SO I KNOW THAT THESE CELLS ARE KIND OF CONTROVERSIAL BECAUSE I THINK THEY ARE PLURIPOTENT, THEY DO NOT PRODUCE TERATOMAS. ONE OF THESE IS PLURIPOTENT TERATOGENESIS MEANS A ONE-TO-ONE DIRECTION. HOWEVER, MY MAJOR GOAL IS TO BRING AWARENESS OF THE EXISTENCE OF THESE CELLS AND TO TRY TO SUPPORT THIS DISCOVER THAT (UNINTELLIGIBLE) EXISTENCE OF PLURIPOTENT STEM CELLS THAT DO NOT PRODUCE TERATOGENESIS AS NATURAL CELLS PRESENT IN THE BODY.

SO MY GRANT WAS REJECTED. I AM THINKING

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TO REAPPLY AGAIN. I THINK WAS PROBABLY THE REVIEWERS WERE NOT AWARE OF THE COMPETENCY OF THESE CELLS, AND THE CRITICS, I THINK THEY WERE NOT FAIR. FOR EXAMPLE, THEY ASKED ME ABOUT THE MECHANISTIC OF ACTION. OKAY. SO I BASICALLY ASK FOR SUPPORT TO THIS UNIQUE OPPORTUNITY (UNINTELLIGIBLE) CLINICAL TRIALS USING THESE PLURIPOTENT STEM CELLS. THANK YOU.

SUPERVISOR SHEEHY: NEXT PLEASE.

DR. NICHOLAS: CORY NICHOLAS, CO-FOUNDER AND CSO OF NEURONA. I'M THE PI ON 10525, WHICH SCORED 80. THIS IS TO DEVELOP A STEM CELL NERVE THERAPY FOR THE TREATMENT OF EPILEPSY. AND I'M HERE TO ADVOCATE FOR EPILEPSY. I THINK WE CAN ALL AGREE THAT EVERY INDICATION IS IMPORTANT, BUT CIRM IS NOT PRESENTLY SUPPORTING ANY EFFORTS TO ADVANCE THERAPIES FOR EPILEPSY.

EPILEPSY IS THE THIRD MOST COMMON DEVASTATING NEUROLOGICAL DISEASE RIGHT BEHIND ALZHEIMER'S DISEASE AND STROKE. OBVIOUSLY A MAJOR HEALTH AND QUALITY OF LIFE CONCERN. HALF A MILLION PEOPLE IN CALIFORNIA SUFFER FROM EPILEPSY, AND ONE-THIRD OF THESE PATIENTS DO NOT RESPOND TO CURRENT ANTI-EPILEPTIC DRUGS, LEAVING THEM WITHOUT ANY GOOD OPTIONS.

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YOU MAY THINK OF THIS AS A MANAGEABLE DISEASE, BUT IT'S NOT. AS LITTLE AS ONE SEIZURE PER YEAR IS ENOUGH TO KEEP SOMEONE FROM DRIVING, FROM HOLDING DOWN A JOB, FROM LIVING INDEPENDENTLY. AND THESE PATIENTS LIVE IN FEAR OF SUDDEN, UNEXPECTED DEATHS FROM EPILEPSY.

WE'VE BEEN WORKING HARD ON THIS THERAPY FOR A VERY LONG TIME. WE HAVE MANY OF THE SAME MERITS AS DISCUSSED BY DR. TUSZYNSKI IN HIS APPLICATION THAT WAS APPROVED FOR SPINAL CORD INJURY. WE'VE BEEN PUBLISHED IN THE MAJOR JOURNALS, AND WE HAVE A TERRIFIC TEAM.

INCLUDING THE ELEVEN APPLICATIONS ALREADY APPROVED FOR FUNDING, IT APPEARS THAT THERE'S A SURPLUS HERE OF AROUND 10 TO $20 MILLION, INCLUDING THE MONEY LEFT OVER FROM THE TRANSLATIONAL BUDGET THAT WASN'T SPENT THIS YEAR.

AND I RESPECTFULLY DISAGREE WITH THE COMMENTS THAT YOU CAN JUST REAPPLY IN MARCH. WHAT YOU'VE JUST APPROVED IS A REDUCED BUDGET NEXT YEAR, AN 80-PERCENT CUT IN THIS VERY COMPETITIVE DISCOVERY ROUND OF GRANTS. SO IT'S NOT GOING TO BE SO EASY NEXT TIME. SO IF YOU HAVE THE MONEY, I ENCOURAGE YOU TO INVEST IN VERY STRONG APPLICATIONS LIKE OURS.

WE HAD A SCORE OF 80, THE SAME SCORE AS

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THE APPLICATION JUST BUMPED UP. IN FACT, WE HAD MORE MEMBERS OF THE GRANTS WORKING GROUP THAT RECOMMENDED OUR APPLICATION FOR FUNDING, ABOUT HALF OF THOSE FOLKS. SO THANK YOU FOR CONSIDERING TO INCLUDE OUR APPLICATION IN THE FUNDING GROUP.

SUPERVISOR SHEEHY: IS THERE ANY MORE PUBLIC COMMENT? YES.

DR. KRIEGSTEIN: ARNOLD KRIEGSTEIN HERE. I'M A PROFESSOR OF NEUROLOGY AT UC SAN FRANCISCO. I'D LIKE TO COMMENT ON WHAT DR. NICHOLAS JUST MENTIONED ABOUT THE EPILEPSY PROPOSAL. THIS PROJECT BEGAN ABOUT A DECADE AGO IN A NUMBER OF ACADEMIC LABS AT UCSF, MYSELF AND OTHER COLLEAGUES, USING INHIBITORY NEURONS AS A POTENTIAL THERAPY FOR FOCAL AND MEDICALLY INTRACTABLE EPILEPSY.

THIS IS A PROJECT THAT WAS FUNDED THROUGH SEVERAL ROUNDS OF CIRM FUNDING. IT LED TO THE DEVELOPMENT OF A HUMAN CELL THAT COULD POTENTIALLY BE A CELL THERAPY FOR THIS DISORDER THAT WAS DONE IN OUR ACADEMIC SETTING, AT WHICH POINT THE PROJECT WAS MATURE ENOUGH TO ACTUALLY TALK ABOUT COMMERCIALIZATION; THAT IS, HOW TO PRODUCE LARGE NUMBERS OF THESE CELLS, TO DO THEM IN A GMP FACILITY, AND SCALE THEM UP TO ACTUALLY START A CLINICAL TRIAL.

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AT THAT POINT WE DECIDED TO FOUND A COMPANY, AND MYSELF, ARTURO ALVAREZ-BUYLLA, AND JOHN RUBENSTEIN, ALONG WITH CORY NICHOLAS CO-FOUNDED THIS START-UP IN ORDER TO DO THAT, IN ORDER TO TRY TO MAKE THIS A TREATMENT THAT WE COULD USE IN A CLINICAL SETTING. THAT'S PROCEEDED OVER THE LAST TWO YEARS EXTREMELY WELL. THE PRODUCT AS IT WAS CALLED IS HUMAN INHIBITORY CORTICAL CELL HAS NOW BEEN DEVELOPED IN HUGE QUANTITIES, AND IT'S SCALABLE, AND IT'S ACCORDING TO THE KIND OF SMALL MOLECULES THAT YOU COULD USE FOR A THERAPEUTIC PRODUCT. WE'RE AT THE THRESHOLD OF DEMONSTRATING CLINICAL EFFICACY WITH THE CELL LINE IN EPILEPSY.

THAT'S WHAT THE PROPOSAL IS ABOUT. AND I JUST WANTED TO MENTION THAT THESE ARE RELATIVELY MODEST AMOUNTS OF MONEY FOR THESE QUEST PROGRAMS THAT CAN HAVE A HUGE IMPACT, IN THIS CASE FOR A DISEASE THAT ISN'T PART OF THE PORTFOLIO RIGHT NOW FOR CIRM. SO I WOULD JUST URGE YOU TO RECONSIDER THE POSSIBILITY OF FUNDING THIS TO GET US PAST THIS IMPORTANT NEXT STEP. THANK YOU.

SUPERVISOR SHEEHY: ANY ADDITIONAL PUBLIC COMMENT? MARIA, WILL YOU CALL THE ROLL.

CHAIRMAN THOMAS: MR. JUELSGAARD HAS A COMMENT.

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DR. JUELSGAARD: SO BEFORE WE VOTE, THIS IS A QUESTION, I THINK, FOR THE PROJECTS GROUP IN TERMS OF THE AMOUNT OF MONEY. SO IF WE APPROVE THE ONES THAT HAVE BEEN MOVED TO TIER I OR IN TIER II MOVED TO TIER I AT THIS POINT, HOW MUCH MONEY WILL WE BE SPENDING VERSUS HOW MUCH MONEY WE HAVE? SO WE STILL HAVE THAT AMOUNT AVAILABLE. GOT IT.

WOULD SENATOR TORRES, I THINK HE MADE THE MOTION, RIGHT?

MR. TORRES: THAT IS CORRECT, SECONDED BY MR. JUELSGAARD.

DR. JUELSGAARD: WOULD YOU ACCEPT A FRIENDLY AMENDMENT?

MR. TORRES: ARE WE GOING TO GO DOWN THIS PATH AGAIN? YOU AND I ALWAYS GO DOWN THE FRIENDLY AMENDMENT PATH. WHAT IS YOUR FRIENDLY AMENDMENT?

DR. JUELSGAARD: IT HAS TO DO WITH THIS EPILEPSY INDICATION.

MR. TORRES: YOU WANT TO ADD IT ON?

DR. JUELSGAARD: YES.

MR. TORRES: THAT WOULD BE A SUBSTITUTE MOTION WHICH THE BODY WOULD HAVE TO APPROVE.

DR. JUELSGAARD: OKAY. I'LL DO IT THAT WAY THEN.

MR. TORRES: I COULD ACCEPT THE FRIENDLY

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AMENDMENT TO SAVE TIME.

MR. TOCHER: COULD YOU REPEAT THAT PLEASE, DR. JUELSGAARD?

DR. JUELSGAARD: YES. I WANT TO, ACCORDING TO SENATOR TORRES, MAKE WHAT IS A SUBSTITUTED AMENDMENT TO THE AMENDMENT THAT'S ON THE FLOOR.

MR. TORRES: I WOULD ACCEPT IT AS A FRIENDLY AMENDMENT, BUT I THINK IT MIGHT BE BETTER IF THE ENTIRE BODY VOTED ON IT.

DR. JUELSGAARD: I AGREE WITH YOU.

MR. TORRES: SO YOUR MOTION CAN BE A SUBSTITUTE MOTION.

DR. JUELSGAARD: SO THIS IS A SUBSTITUTE MOTION TO INCLUDE IN THE TIER I GROUP OR TO MOVE INTO THE TIER I GROUP DISC2 10525, DEVELOPMENT OF A CELLULAR THERAPEUTIC FOR TREATMENT OF EPILEPSY.

DR. HIGGINS: SECOND.

SUPERVISOR SHEEHY: SECOND BY SENATOR TORRES.

MR. TORRES: DR. HIGGINS.

SUPERVISOR SHEEHY: DR. HIGGINS.

MR. TORRES: HE SPOKE BEFORE I COULD.

SUPERVISOR SHEEHY: SO IS THIS ATTACHED TO YOUR MOTION, SENATOR TORRES?

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MR. TORRES: YES, THIS WOULD BE ATTACHED TO THE MAIN MOTION.

SUPERVISOR SHEEHY: OKAY. SO DO WE HAVE PUBLIC COMMENT ON THIS MOTION OR ANY BOARD COMMENT ON THIS MOTION? THEN CAN WE CALL THE ROLL ON THE AMENDMENT TO SENATOR TORRES' MOTION, AND THEN WE'LL TAKE UP SENATOR TORRES' MOTION.

CHAIRMAN THOMAS: DR. DULIEGE HAS A QUESTION AND A COMMENT, MR. SUPERVISOR.

DR. DULIEGE: I WANTED TO ASK STEVE WHY HE MADE SPECIFICALLY THIS AMENDMENT AND HIS IMPETUS FOR HAVING THIS GRANT APPROVED COMPARED TO OTHERS THAT WERE RANKED A LITTLE BIT HIGHER.

DR. JUELSGAARD: CERTAINLY. SO WE JUST ESTABLISHED A PRECEDENT, WHICH I WAS RELUCTANT TO ESTABLISH, OF APPROVING GRANTS THAT HAVE NOT BEEN RECOMMENDED BY THE GWG. SO THAT SUGGESTS THAT WE HAVE A BROADER, MORE PROGRAMMATIC PERSPECTIVE. AND I WAS ALSO THEN PERSUADED BY THE TWO SPEAKERS OF THE NEED IN EPILEPSY AND OF, I THINK, THE POSSIBILITY OF THE APPROACH THAT THEY'RE SUGGESTING. AND WE HAVE THE HEADWAY IN FUNDING, AND I NOTE THAT COMING UP A LITTLE LATER IN THIS DISCUSSION WE'RE GOING TO TALK ABOUT REDUCING. THIS IS QUEST DISCOVERY 2 PROVISIONS. WE'RE GOING TO DECREASE RATHER

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SUBSTANTIALLY, OR THAT'S THE PROPOSAL ANYWAY, THE AMOUNT OF MONEY STARTING NEXT YEAR THAT WE WOULD PROVIDE TO THESE PROGRAMS.

DR. DULIEGE: I APPRECIATE THIS. SO WHY CAN'T WE MAKE A MOTION OF THAT, VOTE ON THIS MOTION SEPARATELY; AND THEN, BASED ON WHETHER IT'S ACCEPTED OR NOT, GO BACK TO SENATOR TORRES' PROPOSAL? THAT SEEMS CLEARER FOR US.

DR. JUELSGAARD: I THINK THAT'S EXACTLY HOW WE'RE GOING TO PROCEED.

CHAIRMAN THOMAS: I THINK MR. TOCHER HAS A COMMENT HERE.

MR. TOCHER: THAT'S RIGHT. THAT'S WHAT I JUST WANT TO CLARIFY. JEFF, THIS WILL BE HANDLED AS A SEPARATE MOTION TO AMEND SENATOR TORRES' MOTION BECAUSE WE HAVE CONFLICTS THAT WE WILL NEED TO TAKE CARE OF IN THIS PARTICULAR MOTION THAT WE CAN TREAT DIFFERENTLY IN THE MORE OMNIBUS MOTION. SO THE VOTE THAT YOU WILL BE ASKED TO TAKE HERE WILL BE TO AMEND SENATOR TORRES' MOTION TO INCLUDE MOVING THE APPLICATION 10525 UP TO TIER I.

MR. TORRES: WELL, JUST LET THE RECORD SHOW THAT I PLAN TO VOTE FOR THIS MOTION EVEN THOUGH MR. JUELSGAARD ABSTAINED FROM MY MOTION.

DR. DULIEGE: QUICKLY HERE, IF WE VOTE YES

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ON THIS, THAT WOULD MEAN THAT WE VOTE YES FOR THIS PARTICULAR GRANT TO BE APPROVED?

SUPERVISOR SHEEHY: YES. IT'S ONLY RELEVANT TO THIS PARTICULAR GRANT ADDED TO TIER I AS PART OF SENATOR TORRES' MOTION. ADDITIONAL BOARD COMMENT?

CHAIRMAN THOMAS: DR. LUBIN HAS A COMMENT.

DR. LUBIN: SO I JUST WAS CURIOUS IF YOU CAN GIVE US SOME -- BECAUSE IT SOUNDS LIKE THE EPILEPSY PROGRAM IS VERY COMPELLING AND PREVIOUS GRANTS WERE SUPPORTED BY CIRM. WHY WASN'T IT IN THE TOP CATEGORY? CAN YOU GIVE US ANY INFORMATION ABOUT WHY --

SUPERVISOR SHEEHY: DO WE HAVE A CONFLICT OF INTEREST HERE?

DR. LUBIN: I'M ASKING A QUESTION. IT IS A CONFLICT BECAUSE I AM REPRESENTING UCSF. I CAN'T ASK A QUESTION. SORRY.

DR. PRIETO: MR. CHAIRMAN, COULD I BRIEFLY ASK WHAT WERE THE GWG CONCERNS ON THIS GRANT?

DR. SAMBRANO: THERE WERE SOME CONCERNS, I THINK, IN SOME WAYS SIMILAR TO THE OTHER THAT WE DISCUSSED, BUT NO MAJOR ISSUES OR FATAL FLAWS IN THIS PROPOSAL. THERE WERE SOME GRANT STRUCTURE ISSUES IN TERMS OF PROVIDING CLARITY FOR REVIEWERS

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TO FULLY APPRECIATE OR UNDERSTAND WHAT THE APPLICANTS WERE TRYING TO GET ACROSS.

THERE WERE SOME CONCERNS RELATED TO THE APPROACH IN TERMS OF, FOR EXAMPLE, THE PRIMARY ENDPOINT OF SEIZURES AND HAVING A LITTLE MORE DEFINITION FROM THE APPLICANT AS TO EXACTLY WHAT TYPE OF SEIZURES THEY WOULD BE STUDYING IN THEIR MODEL, THE EXTENT TO WHICH THIS WOULD BE SIGNIFICANT OR MEANINGFUL AS YOU LOOK FORWARD TOWARDS THE CLINIC. SO THOSE ARE CONCERNS THAT WERE HIGHLIGHTED BY THE GWG.

SUPERVISOR SHEEHY: ANY PUBLIC COMMENT ON THIS, ON MR. JUELSGAARD'S MOTION? MARIA, COULD YOU CALL THE ROLL.

MS. BONNEVILLE: ANNEMARIE DULIEGE.

DR. DULIEGE: NO.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

DR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

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MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: AYE.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: ABSTAIN.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: ABSTAIN.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
SUPERVISOR SHEEHY: NOW WE HAVE SENATOR TORRES' MOTION. DO WE HAVE ANY BOARD DISCUSSION ON SENATOR TORRES' AS AMENDED?
MR. TORRES: WOULD YOU RESTATE THE MOTION PLEASE, MR. CHAIRMAN?
SUPERVISOR SHEEHY: SURE. THIS IS A MOTION TO APPROVE ALL THE APPLICATIONS IN TIER I, INCLUDING APPLICATION 10665 AND APPLICATION 10525,

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FOR FUNDING AND TO NOT APPROVE THE REMAINING APPLICATIONS FOR FUNDING. THAT'S HOW I UNDERSTAND IT. IS THAT CONSISTENT?

MR. TOCHER: IF I COULD JUST REMIND FOLKS WHO ARE VOTING ON THE APPLICATION REVIEW SUBCOMMITTEE THAT MAY HAVE A CONFLICT WITH ANY APPLICATION IN EITHER OF THOSE TIERS TO INDICATE THEIR VOTE EXCEPT AS TO THOSE APPLICATIONS WITH WHICH THEY ARE IN CONFLICT. THANKS, JEFF.

SUPERVISOR SHEEHY: SURE.

AND SO I'M NOT HEARING ANY BOARD COMMENT. IS THERE PUBLIC COMMENT ON THE AMENDED MOTION? MARIA, COULD YOU CALL THE ROLL.

MS. BONNEVILLE: ANNEMARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

DR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

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MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES, EXCEPT FOR THOSE WITH WHICH I'M IN CONFLICT.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: THE MOTION CARRIES.
SUPERVISOR SHEEHY: THANK YOU, MARIA. I BELIEVE, CHAIRMAN THOMAS, THAT CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW SUBCOMMITTEE.
CHAIRMAN THOMAS: THANK YOU, MR. SUPERVISOR. WE'LL TAKE A BRIEF BREAK HERE TO GIVE BETH A BREAK. AND WE'LL RESUME -- AND MR. SENATOR A BREAK TOO -- RESUME IN FIVE TO TEN MINUTES HERE. SO

PLEASE HANG ON.

(A RECESS WAS TAKEN.)

CHAIRMAN THOMAS: AGAIN, EVERYBODY, PLEASE TAKE YOUR SEATS. OKAY. WE ARE RESUMING. WE ARE, FOR A NUMBER OF REASONS, GOING TO TAKE ONE MORE ITEM OUT OF ORDER. AND THAT IS ITEM NO. 7, CONSIDERATION OF CONCEPT PLAN CHANGES TO THE DISCOVERY AND TRANSLATION PROGRAMS. WE HAVE A PRESENTATION BY DR. OLSON.

DR. OLSON: OKAY. CHAIRMAN THOMAS, MEMBERS OF THE BOARD, MEMBERS OF THE PUBLIC, AND TEAM CIRM, FIRST YOU HAVE JUST VOTED RESEARCH FUNDING FOR 2018 FOR THE DISCOVERY AND TRAN PROGRAMS. THANK YOU.

WHAT I WOULD LIKE TO DISCUSS WITH YOU NOW ARE OUR PROPOSALS TO WHAT WE WOULD LIKE TO DO TO MAXIMIZE THAT FUNDING TO BETTER SERVE OUR MISSION, AND THESE DO INVOLVE CONCEPT CHANGES. SO I'D LIKE TO START WITH THE DISCOVERY PROGRAM.

SO THE 2018 DISCOVERY BUDGET THAT YOU JUST APPROVED IS $10 MILLION. AND AS WAS NOTED BY A MEMBER OF THE PUBLIC BEFORE AND NOTED HERE AGAIN, IT IS DOWN FROM 52 MILLION IN THE PREVIOUS YEAR. WHAT WE WOULD LIKE TO RECOMMEND IS THAT THAT FUNDING BE FOCUSED ON THE DISC2, THE QUEST PROGRAM; AND THOSE

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ARE THE APPLICATIONS THAT THE APPLICATION REVIEW SUBCOMMITTEE JUST ACTUALLY FUNDED IN THE LATEST ROUND.

THE RATIONALE FOR FOCUSING ON THE QUEST PROGRAM IS IT IS A DIRECT FEED INTO OUR EARLY TRANSLATION PROGRAM. SO THE GOAL IS A CANDIDATE TO MOVE INTO TRANSLATION. AND THAT PROGRAM FUNDS THOSE ACTIVITIES THAT ENABLE THAT TO HAPPEN.

IT IS, AS NOTED AGAIN BY GIL, IT IS THE WORKHORSE OF OUR DISCOVERY PROGRAM. IT IS OUR MOST POPULAR PROGRAM. IN THE TWO YEARS THUS FAR THAT PROGRAM HAS BEEN ONGOING, THERE HAVE BEEN FOUR ROUNDS, AND WE HAVE RECEIVED 321 APPLICATIONS WHICH THE GRANTS WORKING GROUP HAS LOOKED AT IN ONE WAY OR ANOTHER.

SO IT ALSO LEVERAGES PAST INVESTMENTS IN BASIC RESEARCH. SO YOU HEARD FROM DR. KREIGSTEIN ABOUT HOW THAT CIRM HAD FUNDED SOME BASIC BIOLOGY PROGRAMS THAT ESSENTIALLY LED TO THEM BELIEVING THEY ARE VERY NEAR HAVING A CANDIDATE TO MOVE INTO TRANSLATION AND INTO CLINICAL DEVELOPMENT. SO PREVIOUS CIRM WORK IS THE ONE THAT A LOT OF IT GOES TO QUEST AND SAYING WE'RE READY TO MOVE FORWARD.

HOWEVER, WHAT WE WOULD LIKE TO DO IS TO MAXIMIZE THIS BUDGET ALLOCATION OF $10 MILLION. WE

WOULD PROPOSE A REDUCTION IN THE DIRECT PROGRAM COST CAPS TO MAXIMIZE THE BUDGET ALLOCATION AND ALLOW US TO MAINTAIN SOMEWHAT OF A PIPELINE IN THIS AREA. SO THESE ARE JUST A FEW POINTS ABOUT THIS PROGRAM.

CURRENTLY, SO IN THE ROUND THAT YOU JUST DID AND FOR THE LAST TWO YEARS, CURRENTLY CAPS DIRECT PROJECT COSTS AS FOLLOWS. IT'S ALWAYS UP TO, AN APPLICANT CAN ALWAYS PROPOSE LESS, BUT IT'S UP TO $1.4 MILLION FOR THERAPEUTIC CANDIDATE DISCOVERY. IT'S UP TO $0.7 MILLION FOR A MEDICAL DEVICE, DIAGNOSTIC, OR TOOL AND TECHNOLOGY CANDIDATE DISCOVERY. AND THE RATIONALE FOR THAT DISCREPANCY IS THAT, IN FACT, IT IS GENERALLY MORE EXPENSIVE TO DO THOSE ACTIVITIES TO ACHIEVE A THERAPEUTIC CANDIDATE AS IT IS TO ACHIEVE THOSE ACTIVITIES TO ACHIEVE A DEVICE, A TECHNOLOGY, OR A DIAGNOSTIC PROTOTYPE READY TO MOVE INTO DEVELOPMENT.

SO WHAT WE ARE RECOMMENDING FOR CONSIDERATION BY THIS BOARD IS THAT WE REDUCE THE DIRECT PROJECT COST CAPS AS FOLLOWS: FROM 1.4 TO 0.9 MILLION FOR THERAPEUTIC CANDIDATE DISCOVERY AND FROM 0.7 TO 0.5 MILLION FOR MEDICAL DEVICE, DIAGNOSTIC, AND TOOL AND TECHNOLOGY CANDIDATE DISCOVERY. THIS IS PRETTY MUCH AN ACROSS-THE-BOARD REDUCTION IN DIRECT PROJECT COSTS OF BETWEEN 30 AND

36 PERCENT FOR ALL OF THESE DIFFERENT PROGRAM TYPES.

I WILL POINT OUT TO YOU THAT BY AND LARGE THE BULK OF OUR APPLICATIONS OF THOSE 321 APPLICATIONS ARE THERAPEUTIC CANDIDATE DISCOVERY. SO, IN FACT, THAT ONE WE'RE PROPOSING A LITTLE BIT MORE OF A REDUCTION.

THE RATIONALE IS TO MAXIMIZE THIS REDUCED DISCOVERY BUDGET WHILE MAINTAINING A QUALITY PIPELINE. SO WE ANTICIPATE THAT WE THINK THAT WE CAN FUND WITH THESE KINDS OF CAPS BETWEEN SEVEN AND EIGHT PROJECTS NEXT YEAR. SO IT KEEPS OUR PIPELINE GOING, AND IT ALLOWS QUALITY APPLICATIONS TO CONTINUE TO MOVE FORWARD.

I'D THEN ALSO LIKE TO TALK ABOUT ANOTHER PROPOSED CONCEPT CHANGE, AND THIS IS TO THE TRAN PROGRAM. SO YOU'VE HEARD ABOUT TRAN AND CLIN1 ARE THE VALLEY OF DEATH. THIS IS THE STAGE THAT IS VERY DIFFICULT TO GET RESEARCH FUNDING. SO TRAN IS ESSENTIALLY EARLY DEVELOPMENT. THE ENTRY IS A CANDIDATE THAT'S READY TO MOVE INTO DEVELOPMENT. THE GOAL OF THE PROGRAM IS A PRE-IND MEETING, WHICH IS WHAT YOU HOLD BEFORE YOU DO YOUR PIVOTAL STUDIES AND READY YOURSELF TO MOVE INTO THE CLINIC. SO WE CONSIDER THIS A PRETTY CRITICAL PROGRAM.

THE TRAN CONCEPT PLAN CURRENTLY DOES NOT

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ALLOW NON-CALIFORNIA APPLICANTS TO APPLY FOR FUNDING TO CONDUCT RESEARCH IN CALIFORNIA. THIS CHANGE TO THE CONCEPT PLAN WAS IMPLEMENTED BY THE BOARD IN DECEMBER LAST YEAR FOR BOTH THE DISC AND THE TRAN PROGRAMS. WHAT WE ARE ASKING THE BOARD TO RECONSIDER NOW IS FOR THE TRAN PROGRAM ONLY TO REINSTATE ELIGIBILITY FOR NON-CALIFORNIA ORGANIZATIONS.

THE RATIONALE FOR THAT IS WE WOULD LIKE TO INCREASE OUR POOL OF APPLICANTS AND, THEREFORE, THE OPPORTUNITY FOR MORE QUALITY AWARDS. SO I WOULD POINT OUT THAT THIS YEAR, 2017, COMPARED TO LAST YEAR, 2016, WE HAD A 33-PERCENT REDUCTION IN THE NUMBER OF APPLICATIONS THAT WENT IN FRONT OF THE GRANTS WORKING GROUP, AND WE HAD A 50-PERCENT REDUCTION IN THE NUMBER OF AWARDS MADE COMPARED TO 2016. SO WE THINK THAT BY OPENING IT UP WE WILL GET MORE APPLICATIONS AND THEN PRESUMABLY POTENTIALLY MORE QUALITY PROGRAMS TO FUND.

AS CIRM RECOGNITION HAS GROWN, I'M NOT SURE YOU'RE AWARE, BUT AS YOU CAN PERHAPS TELL FROM DR. MILLAN'S PRESENTATION, CIRM'S BRAND, IF YOU LIKE IT IN TALKING MARKETING, IS ACTUALLY BECOMING QUITE WELL-KNOWN, AND SO WE'RE GETTING A LOT OF EXTERNAL INTEREST IN THIS PROGRAM AS WELL.

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THEN, IN ADDITION, FOR THOSE OUT-OF-STATE APPLICATIONS, IT WOULD BE AN OPPORTUNITY TO ENGAGE WITH US EARLY IN THE DEVELOPMENT PIPELINE IN ORDER TO HELP THEM BE MORE SUCCESSFUL AT THE LATER STAGES OF CIRM'S FUNDING WHERE THEY ARE ELIGIBLE. SO WE WOULD ALSO, AS WITH THE CLIN PROGRAMS, THE CIRM FUNDING WOULD ONLY BE FOR RESEARCH ACTIVITIES THAT WERE CONDUCTED IN CALIFORNIA OR THAT ARE DIRECTLY REQUIRED TO SUPPORT THE RESEARCH CONDUCTED IN CALIFORNIA. SO, AGAIN, WE FOCUS ON FUNDING RESEARCH THAT'S DONE IN CALIFORNIA.

SO THE REQUESTED ACTIONS OF THE BOARD IS WE WOULD REQUEST THAT YOU APPROVE THE PROPOSED AMENDMENT TO THE DISC CONCEPT PLAN TO ESSENTIALLY PUT IN CAPS, LOWERED CAPS, ON THE DIRECT PROJECT COSTS AND TO APPROVE THE PROPOSED AMENDMENT TO THE TRAN CONCEPT PLAN, WHICH WOULD BE TO ALLOW OUT-OF-STATE APPLICANTS TO BE ELIGIBLE TO APPLY.

I'M HAPPY TO ANSWER ANY QUESTIONS THAT YOU MAY HAVE AND THANK YOU.

CHAIRMAN THOMAS: SO BEFORE WE PROCEED TO ANY MOTIONS, ARE THERE QUESTIONS BY MEMBERS OF THE BOARD?

DR. BERGLUND: SO I'M WONDERING, YOU MENTIONED THERE WAS A 50-PERCENT REDUCTION OF FUNDED

PROPOSALS IN THE TRAN CONCEPT. CAN YOU TELL US HOW MANY WERE FUNDED FROM OUTSIDE CALIFORNIA IN THE YEAR 2016?

DR. OLSON: FROM OUTSIDE OF CALIFORNIA, IN 2016, ONE WAS FUNDED.

DR. BERGLUND: AND IN 2017? AND BEFORE --

DR. OLSON: THEY WEREN'T ELIGIBLE IN 2017.

DR. BERGLUND: OKAY. SO THE ONE.

DR. OLSON: SO ONE IN 2016 WAS FUNDED.

DR. BERGLUND: OUT OF HOW MANY?

DR. OLSON: OUT OF ABOUT FOUR APPLICANTS, BUT, AGAIN, THEY DIDN'T HAVE ANY OPPORTUNITY TO REAPPLY. AND AS I NOTED, OUR NAME RECOGNITION IS GROWING AND EXTERNAL INTEREST IS GROWING AMONG SOME PEOPLE THAT WE ACTUALLY ARE EXCITED ABOUT.

CHAIRMAN THOMAS: OTHER QUESTIONS OF DR. OLSON?

MS. WINOKUR: I WOULD ASK THAT IN ANY MOTION THAT WE CHANGE THE WORDING ON THE TRAN CONCEPT PLAN IT'S AVAILABLE TO OUT OF CALIFORNIANS BUT FOR RESEARCH IN CALIFORNIA.

DR. OLSON: YES. AS I NOTE HERE, CIRM FUNDS WOULD ONLY FUND RESEARCH -- OH, YOU WANT IT TO BE REQUIRED AS PART OF THE MOTION. OKAY.

CHAIRMAN THOMAS: LET'S TAKE THESE ONE AT

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A TIME HERE. YOU HAVE ANOTHER COMMENT, DR. DULIEGE?

DR. DULIEGE: JUST WANTED TO MAKE A COMMENT. I REALLY APPRECIATE YOUR PROPOSAL BOTH WAYS. ON ONE HAND, IT'S FINANCIALLY MORE CONSERVATIVE AND WE NEED TO DO SO; AND ON THE OTHER HAND, IT WILL ALLOW TO INCREASE THE POOL OF HIGH QUALITY PROPOSALS. SO THAT MAKES A LOT OF SENSE TO ME.

DR. OLSON: THANK YOU.

CHAIRMAN THOMAS: ANY QUESTIONS? WE HAVE A COUPLE MORE HERE. DR. STEWARD AND THEN MR. JUELSGAARD.

DR. STEWARD: SO JUST TO GET A SENSE OF WHAT THIS MEANS IN TERMS OF PERCENT FUNDING, HOW MANY OF THE DISC APPLICATIONS HAVE WE BEEN GETTING OVER THE PAST COUPLE OF YEARS? DO YOU HAVE A SENSE OF THAT?

DR. OLSON: OF THE QUEST APPLICATIONS, WE HAVE RECEIVED 321 IN THE FOUR ROUNDS THAT HAVE OCCURRED. NOW, AS YOU RECALL, THE GRANTS WORKING GROUP GOES THROUGH A TWO-STAGE REVIEW PROCESS UNDER THOSE CIRCUMSTANCES. YES, IT IS OUR MOST POPULAR PROGRAM.

DR. STEWARD: AND OF THOSE FOUR REVIEWS, WAS THAT ALL IN -- THAT'S SPREAD OUT OVER, WHAT, TWO

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YEARS?

DR. OLSON: YES. THOSE FOUR REVIEWS WERE OVER A TWO-YEAR PERIOD THAT THIS PROGRAM HAS BEEN IN OPERATION.

DR. STEWARD: SO JUST TO POINT OUT THE OBVIOUS, WHEN YOU DO THE MATH, THAT MEANS ABOUT 150 PER YEAR. YOU'RE TALKING ABOUT FUNDING MAYBE EIGHT TO TEN. SO OUR SUCCESS RATE FOR FUNDED PROPOSALS IS GOING TO BE LESS THAN NIH BY A GOOD BIT, WHICH IS --

DR. OLSON: YOU KNOW, I MEAN --

DR. STEWARD: I'M JUST POINTING IT OUT.

DR. OLSON: AND WE ALL RECOGNIZE THAT, AND THERE JUST IS THE REALITY OF WHERE WE ARE IN OUR FUNDING CYCLE.

DR. STEWARD: I TOTALLY APPRECIATE THAT. I WANTED TO SAY IT OUT LOUD, THOUGH, SO THAT WE UNDERSTAND WHERE WE ARE. AND I JUST ALSO WANT TO SAY OUT LOUD THAT I THINK THAT WE DO NEED TO PAY VERY CAREFUL ATTENTION TO THAT ENTRY STAGE BECAUSE I THINK THERE ARE STILL SOME GREAT THINGS TO BE DISCOVERED AND TO COME INTO THE PIPELINE. IT'S REALLY UNFORTUNATE THAT WE'RE CLOSING DOWN THIS VALVE RIGHT NOW. I UNDERSTAND THE NEED FOR IT, BUT I JUST WANT TO SAY THAT OUT LOUD. THANK YOU.

DR. JUELSGAARD: DR. OLSON, I'D LIKE TO

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JUST TURN TO THE TRAN PRESENTATION. SO YOU INDICATED THAT RECENTLY WE VOTED, THIS GROUP VOTED TO NOT HAVE NON-CALIFORNIA INSTITUTIONS PARTICIPATE, AND NOW WE'RE RECOMMENDING TO TURN RIGHT AROUND AND DO THE OPPOSITE. REMIND ME, WHAT WERE THE ORIGINAL RECOMMENDATIONS GOING BACK TO THE VOTE TO NOT ALLOW NON-CALIFORNIA ORGANIZATIONS TO PARTICIPATE? WHAT WAS THE RATIONALE THAT WENT BEHIND DOING THAT?

DR. OLSON: THANK YOU FOR THAT QUESTION. IN THE FIRST YEAR, IN 2016, WHICH WAS THE FIRST YEAR OF THE TRAN PROGRAM, WE HAD 51 APPLICATIONS THAT WERE REVIEWED, AND 12 WERE FUNDED. THIS YEAR, 2017, WE ONLY HAD 34 APPLICATIONS THAT WE RECEIVED, SO A REDUCTION, 33 PERCENT, AND WE ONLY FUNDED SIX. SO A 50-PERCENT REDUCTION IN THE NUMBER OF AWARDS.

SO BASICALLY WHAT WE'RE TRYING TO DO IS BROADEN THE APPLICANT POOL WITH THE GOAL OF MORE HIGH QUALITY, FUNDABLE APPLICATIONS.

DR. GASSON: I JUST WANTED TO FOLLOW UP ON WHAT DR. STEWARD SAID. AND I UNDERSTAND WHY WE'RE DOING WHAT WE'RE DOING, AND I FULLY SUPPORT IT. BUT IN ADDITION TO THE SUCCESS RATE BEING LOW, THOSE OF US WHO HAVE BEEN ON STUDY SECTIONS FOR NIH REALIZE HOW DIFFICULT IT IS TO PICK THE RIGHT, IF YOU WILL, TEN APPLICATIONS OUT OF A HUNDRED OR MORE. AND

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UNFORTUNATELY I'M MAKING A COMMENT WITHOUT HAVING A SOLUTION OR A PROPOSAL, WHICH I HATE TO DO, BUT I'M JUST FOLLOWING UP ON WHAT DR. STEWARD SAID.

CHAIRMAN THOMAS: OTHER QUESTIONS OR COMMENTS BY MEMBERS OF THE BOARD ON THE PHONE BEFORE WE PROCEED TO ANY MOTIONS? HEARING NONE, DO I HEAR A MOTION TO APPROVE THE PROPOSED AMENDMENT TO THE DISC CONCEPT PLAN?

DR. MARTIN: SO MOVED.

CHAIRMAN THOMAS: MOVED BY DR. MARTIN. SECONDED BY --

DR. HIGGINS: SECOND.

CHAIRMAN THOMAS: -- DR. HIGGINS. ANY DISCUSSION BY MEMBERS OF THE BOARD? ANYBODY ON THE PHONE? ANY PUBLIC COMMENT? HEARING NONE, WE'LL PROCEED TO A VOICE VOTE PLUS ROLL OF THOSE ON THE PHONE. ALL IN THE ROOM IN FAVOR OF THIS MOTION PLEASE SIGNIFY BY SAYING AYE. OPPOSED? ABSTAIN? MARIA, PLEASE CALL THE ROLL OF THOSE ON THE PHONE.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: YES.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: JACK DIXON.

DR. DIXON: NO.

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MS. BONNEVILLE: LAUREN MILLER. JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: YES.

MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU. DO I HAVE A MOTION TO APPROVE THE PROPOSED AMENDMENT TO THE TRAN CONCEPT PLAN?

DR. HIGGINS: SO MOVED.

CHAIRMAN THOMAS: MOVED BY DR. HIGGINS. SECONDED BY --

DR. PRIETO: SECOND.

CHAIRMAN THOMAS: -- DR. PRIETO.

ANY COMMENTS BY MEMBERS OF THE BOARD EITHER IN THE ROOM OR ON THE PHONE? ANY PUBLIC COMMENT? WE DO HAVE PUBLIC COMMENT.

DR. CHIU: I TOTALLY UNDERSTAND THAT CIRM WANTS TO FUND THE VERY BEST PROPOSALS TO THE EXTENT POSSIBLE. I ALSO WANT TO REMEMBER THAT YOU MADE A GREAT EFFORT TO RECRUIT REALLY GREAT SCIENTISTS INTO

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CALIFORNIA, WHICH MAKES THE STEM CELL COMMUNITY HERE FANTASTIC. AND TO SUPPORT THEM, TRAN IS SUCH A POWERFUL MECHANISM TO HAVE THEIR IDEAS MOVE INTO THE CLINIC. AND THIS IS A PROTECTED SPACE FOR THOSE WHO CHOSE TO COME TO CALIFORNIA AND CALIFORNIA INSTITUTIONS TO DO THE WORK IN CALIFORNIA AND MAKE THIS STATE THE EPICENTER OF STEM CELL THERAPY.

I JUST FEEL THAT BY OPENING IT EVEN THIS WAY TO THOSE OUTSIDE, THAT YOU ARE REDUCING THE POSSIBILITIES FOR CALIFORNIA SCIENTISTS. THIS IS A VERY TOUGH MECHANISM, AND CIRM STAFF GIVES A LOT OF HELP TO MAKE SURE PEOPLE ARE MOVING IN THE RIGHT DIRECTION. AND GIVEN THE TIGHT FUNDING NOW, I JUST FEEL IN MY HEART THAT PERHAPS IT SHOULD STILL BE CONSERVED FOR CALIFORNIAN SCIENTISTS AND INVESTIGATORS AND COMPANIES. AND THAT IF THEY REALLY WANT TO DO THIS, THEY SHOULD MOVE HERE AND NOT OPEN THE DOOR TO THOSE OUTSIDE JUST BECAUSE THEY WANT TO DO A FEW EXPERIMENTS OR USE A FEW FACILITIES INSIDE CALIFORNIA. THAT'S JUST MY PERSONAL VIEW. THANK YOU.

CHAIRMAN THOMAS: ADDITIONAL PUBLIC COMMENT?

DR. LORING: THANKS, ARLENE. THAT'S EXACTLY WHAT I WAS THINKING. I WANT TO SECOND YOUR

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SUGGESTION.

CALIFORNIA, THIS WAS ALWAYS DESIGNED TO MAKE CALIFORNIA THE CENTER OF STEM CELL THERAPY. AND I KNOW A LOT OF PEOPLE OUTSIDE OF CALIFORNIA WOULD LOVE TO USE CIRM AS A FUNDING SOURCE, AS AN ADDITIONAL FUNDING SOURCE, BUT I THINK THAT WHAT HAS MADE CIRM SO GREAT AND CALIFORNIA SO GREAT AT THIS IS THE RESTRICTION TO PEOPLE WHO WANTED TO MOVE TO CALIFORNIA IF THEY REALLY, REALLY WANTED A GRANT LIKE THIS. THANKS.

DR. BLUMENTHAL: I'LL SIMPLY SECOND ALL OF THOSE COMMENTS WHICH I THINK ARE REALLY GOOD.

DR. OLSON: SO I'D JUST LIKE TO MAKE TWO COMMENTS. MY FIRST COMMENT IS THAT LAST YEAR, 2017, THE PROGRAM HAD A BUDGET THIS BOARD APPROVED FOR THE 2017 TRAN BUDGET, $45 MILLION. THE BOARD MADE -- THE GRANTS WORKING GROUP RECOMMENDED AND THE BOARD APPROVED AWARDS TOTALING $24 MILLION, HALF OF THE ALLOCATED BUDGET.

THE SECOND POINT I WOULD LIKE TO MAKE, AS THE BOARD, THE BOARD ALWAYS HAS THE PROGRAMMATIC OPPORTUNITY TO PREFER IN A COMPETITIVE SITUATION A CALIFORNIA ORGANIZATION OVER A NON-CALIFORNIA ORGANIZATION. THAT IS A PROGRAMMATIC CONSIDERATION THAT FALLS RIGHT WITHIN THE BOARD'S BAILIWICK.

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DR. MALKAS: CAN I SPEAK?

CHAIRMAN THOMAS: OF COURSE. DR. MALKAS.

DR. MALKAS: SO WHAT DO WE TELL THE CITIZENS OF CALIFORNIA?

DR. OLSON: WE TELL THEM THAT WE ARE FUNDING ACTIVITIES CONDUCTED IN CALIFORNIA. THAT'S WHAT WE TELL THEM, THAT THE ACTIVITIES FUNDED ARE CONDUCTED IN CALIFORNIA, FUND CALIFORNIA INVESTIGATORS, CALIFORNIA SUPPORT PEOPLE, CALIFORNIA INSTITUTIONS. SO THAT'S WHAT WE TELL THEM.

AND ON THE BROADER LEVEL, WE TELL THEM THAT THEY'RE FOR THERAPIES THAT WE HOPE WILL BENEFIT PATIENTS, WILL MOVE FORWARD AND WILL BENEFIT PATIENTS EVERYWHERE.

MR. TOCHER: I JUST WANT ADD A LITTLE ADDITIONAL DETAIL TO PAT'S ANSWER. AS THE LANGUAGE INDICATES, AND THIS IS SIMPLY RESTORING THE LANGUAGE THAT WAS PART OF THE 2016 PROGRAM WHEN WE ALLOWED OUT-OF-STATE APPLICANTS, THE ALLOWABLE COSTS FOR A NON-CALIFORNIA ORGANIZATION INCLUDE THE COST OF RESEARCH ACTIVITIES THAT ARE CONDUCTED IN THE STATE, BUT ALSO FOR THE SHARE OF COSTS OF RESEARCH ACTIVITIES CONDUCTED OUTSIDE OF CALIFORNIA THAT ARE DIRECTLY REQUIRED TO SUPPORT THE CLINICAL RESEARCH THAT'S CONDUCTED IN CALIFORNIA.

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SO WE'VE USED, AS AN EXAMPLE, THE CASE WHERE IF THERE WAS AN ANIMAL STUDY BEING CONDUCTED IN CALIFORNIA, THE STUDY ITSELF WOULD BE PAID FOR BECAUSE THOSE RESEARCH ACTIVITIES ARE CONDUCTED IN THE STATE. BUT IN ADDITION, IF THERE WERE CELLS THAT NEED TO BE MANUFACTURED FOR USE IN THE TRIAL, IT WOULD BE A PRO RATA SHARE OF THOSE CELLS AND THE COST FOR THOSE CELLS THAT WOULD BE USED IN THE CALIFORNIA STUDY.

CHAIRMAN THOMAS: MR. SUPERVISOR, I UNDERSTAND YOU HAVE A COMMENT.

SUPERVISOR SHEEHY: YEAH. I ACTUALLY TEND TO AGREE WITH THE PUBLIC COMMENTERS. I THINK IT'S IMPORTANT THAT THE FUNDING STAY IN CALIFORNIA TO THE LARGEST DEGREE POSSIBLE. AND IF IT WAS POSSIBLE TO MAKE AN AMENDMENT TO HAVE THIS LIMITED TO CALIFORNIA, I WOULD MAKE THAT MOTION.

CHAIRMAN THOMAS: MR. TOCHER.

MR. TOCHER: WE ALREADY HAVE A MOTION ON THE TABLE TO APPROVE THE CHANGES, AND THAT WAS MADE BY DR. HIGGINS AND SECONDED BY DR. PRIETO.

SUPERVISOR SHEEHY: SORRY. I APOLOGIZE.

CHAIRMAN THOMAS: DR. MARTIN, DO YOU HAVE A COMMENT?

I WOULD JUST LIKE TO MAKE A COMMENT MYSELF

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HERE, WHICH IS THAT, IN MAKING THIS ELIGIBLE TO OUT-OF-STATE APPLICANTS WHO HAVE COMPONENTS THAT ARE WITHIN CALIFORNIA, THIS IS BRINGING IT INTO CONSISTENCY WITH OUR CLIN2 PROGRAMS WHICH HAVE THAT AVAILABILITY.

SECONDLY, I WOULD SAY HERE THAT GIVEN THE TREND THAT DR. OLSON HAS IDENTIFIED AND THE MISSION OF CIRM TO FUND THE BEST POSSIBLE PROJECTS, IF THERE HAPPENED TO BE SOME FROM OUT OF STATE THAT HAVE LEGITIMATE CALIFORNIA COMPONENTS, THAT WE CAN FUND IF RECOMMENDED BY THE GWG. IN MY PERSONAL OPINION, TO ADVANCE OUR MISSION, THAT IS A GOOD THING TO DO.

DR. STEWARD.

DR. STEWARD: TWO COMMENTS. I DO THINK THIS IS DIFFERENT THAN THE CLINICAL PROGRAMS. IN THE CLINICAL PROGRAMS, YOU CAN IMAGINE SITUATIONS WHERE IT WOULD BE VERY DIFFICULT TO PUT TOGETHER A CLINICAL TRIAL BECAUSE THE NUMBER OF PATIENTS IS SMALL, AND SO YOU'RE GOING TO NEED TO RECRUIT EXPERTISE FROM OUTSIDE THE STATE OF CALIFORNIA TO ACTUALLY MOVE THE TRIAL ALONG. I THINK THAT'S HARDER TO MAKE IN THE TRAN COMPONENT OF THE WORK BECAUSE IT'S JUST A LOT SIMPLER BECAUSE OF WHAT YOU'RE TRYING TO DO. THAT'S ONE COMMENT.

THE SECOND COMMENT IS THAT, ALTHOUGH I

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UNDERSTAND THE EXPLANATION THAT THE MONEY THAT WOULD BE SPENT WOULD BE SPENT IN CALIFORNIA EVEN THOUGH IT WAS AWARDED SOMEWHERE ELSE, I CAN'T EVEN EXPLAIN THAT TO MYSELF. AND I THINK THAT'S A HARD CONCEPT TO EXPLAIN TO THE CITIZENS OF CALIFORNIA EVEN THOUGH IT'S SORT OF UNDERSTANDABLE WHEN YOU REALLY DIG DEEP.

SO THOSE ARE THINGS THAT BOTHER ME ABOUT THIS. AND JUST THE OTHER THING I'LL ASK A PROCEDURAL QUESTION, WHICH IS THAT IF WE WANTED TO MOVE TO JEFF'S MOTION, THEN I GUESS THE OPTION WOULD BE TO VOTE NO ON THIS AND TO TAKE UP A SECOND MOTION, WHICH IS WHERE I'M INCLINED TO GO RIGHT NOW.

MR. TORRES: OR YOU CAN PROVIDE A SUBSTITUTE.

CHAIRMAN THOMAS: BEFORE WE ASK MR. TOCHER TO ADDRESS THAT COMMENT AND THE VARIETY OF OPTIONS THAT HAVE BEEN PUT ON THE TABLE HERE, DR. OLSON, WITH RESPECT TO DR. STEWARD'S QUESTION ABOUT SORT OF HOW THAT WOULD WORK, WRAP HIS HANDS AROUND HOW YOU COULD HAVE A TRAN WITH A CALIFORNIA COMPONENT, PERHAPS YOU COULD ADDRESS THAT JUST SO THE BOARD WOULD UNDERSTAND A FOR INSTANCE OF HOW THAT MIGHT WORK.

DR. OLSON: OKAY. SO, FOR EXAMPLE, IN AN

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AWARD THAT WAS -- AN EXAMPLE OF AN AWARD WOULD BE YOU CONTRACT WITH A LAB AT ANY OF OUR MANY GREAT INSTITUTIONS TO DO ANIMAL MODELS, YOU CAN TRACK TO DO MANUFACTURING AT ANY OF OUR GMP FACILITIES WITHIN THE STATE, YOU HAVE A COLLABORATOR WHO'S AN EXPERT IN CERTAIN MECHANISTIC AND/OR MODELS, AND SO THAT WOULD BE HOW IT WOULD WORK. THOSE ARE EXAMPLES.

CHAIRMAN THOMAS: I'D LIKE MR. TOCHER TO RESPOND PROCEDURALLY HERE HOW WE WOULD GO.

MR. TOCHER: IT SOUNDS LIKE THE FRIENDLY OR UNFRIENDLY AMENDMENT WOULD BE THE OPPOSITE OF WHAT THE MOTION IS THAT'S ON THE TABLE. I WOULD RECOMMEND YOU JUST PROCEED, UNLESS IT'S WITHDRAWN, THAT YOU JUST PROCEED WITH THE MOTION THAT'S ON THE TABLE. AND IF IT IS DEFEATED, THEN MAKE A SUBSEQUENT MOTION.

CHAIRMAN THOMAS: THANK YOU. DR. MILLAN AND THEN WE'LL GET TO A COUPLE BOARD MEMBER COMMENTS.

DR. MILLAN: SO I JUST WANTED TO ADD TO WHAT DR. OLSON'S RESPONSE WAS TO YOUR QUESTION, CHAIRMAN THOMAS OR DR. STEWARD, IN TERMS OF SCENARIOS.

SO AS I HAD MENTIONED IN MY TALK, WE WERE AT AN FDA/NIH WORKSHOP LAST WEEK. AND ONE OF THE

BIG CHALLENGES IS STANDARDIZATION OF THINGS SUCH AS HOW DO YOU STANDARDIZE IPSC PRODUCTION, FOR INSTANCE, OR HOW DO YOU GET SOME OF THESE DISCOVERIES SCALED UP, AND HOW DO WE GET AGREEMENT IN THE ENTIRE COMMUNITY OF WHAT MAKES A PRODUCT READY TO GO INTO PATIENTS. AND THOSE TYPES OF CHALLENGES THAT WE FACE WE CAN'T ADDRESS IN A SINGLE STATE. AND THAT'S NO. 1.

NO. 2, AS THIS FIELD IS MATURING, WE HAVE BEEN IN CONVERSATIONS WITH A VARIETY OF INVESTIGATORS OUTSIDE OF CALIFORNIA THAT HAVE REAL POTENTIALLY TRANSFORMATIVE TECHNOLOGIES THAT THEY CAN BRING TO BEAR TO THE PROBLEMS THAT WE HAVE, INCLUDING BRINGING THEIR IP IN SO THAT IT CAN BE DEVELOPED IN CALIFORNIA AND ACTUALLY DRAW FROM THE EXPERTISE IN CALIFORNIA IN TERMS OF MANUFACTURING AND THE CONDUCT OF THESE CLINICAL TRIALS AS WELL AS CLINICAL DEVELOPMENT ACTIVITIES.

SO IT'S NOT THAT WE'RE OPENING UP AND WE'RE LOSING THINGS. THE OPPORTUNITY HERE IS TO GROW WHAT WE'VE ALREADY BUILT. AND I THINK IT'S SO CRITICAL AT THIS STAGE AND WHERE WE ARE IN THIS FIELD THAT WE PUT ALL THE FIREPOWER BEHIND IT. WE HAVE AN AMAZING ECOSYSTEM WITHIN CALIFORNIA NOW THAT WE'VE BUILT UP, BUT THERE'S MORE THAT NEEDS TO COME

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IN. AND THIS IS GOING TO TAKE ALL THE EXPERTISE AROUND THE COUNTRY AND INTERNATIONALLY TO TACKLE THESE PROBLEMS.

CHAIRMAN THOMAS: WE HAVE A NUMBER OF COMMENTS FROM MEMBERS OF THE BOARD. START WITH DR. MELMED.

DR. MELMED: I CAN FORESEE THIS DEBATE BEING A DISTRACTION WHEN WE'RE GOING FOR OUR BALLOT MEASURE. I THINK WE'RE FRAUGHT WITH UNNECESSARY DISTRACTION. AND IF WE CAN'T EXPLAIN IT TO OURSELVES IN THIS ROOM, IT'S GOING TO BE SO CHALLENGING AND UNFORTUNATE TO GET INTO THE PUBLIC DEBATE. SO THAT'S WHY I THINK THAT I WOULD SUPPORT THE REVERSE RESOLUTION.

CHAIRMAN THOMAS: DR. MARTIN.

DR. MARTIN: I WASN'T HERE, BUT I CONSIDER THE 2016 CLOSING TO A RESTRICTION TO CALIFORNIA APPLICANTS AN EXPERIMENT. AND I THINK THE RESULTS OF THE EXPERIMENT YOU GAVE US, PAT, AND THAT IS THAT THERE WAS NOT A FIXED PERCENTAGE OF THE APPLICANTS WHO WERE FUNDED AND THERE WAS NOT A FIXED EXPENDITURE FOR THE APPLICANTS. IT WAS A QUALITY JUDGMENT THAT REDUCED THE NUMBER OF APPLICANTS TO 50 PERCENT. AND, THEREFORE, NONE OF THE CALIFORNIA APPLICANTS WERE DISADVANTAGED BY ITS BEING OPEN

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PRIOR TO THAT OR BY CLOSING IT OUTSIDE. THEY WERE NOT ADVANTAGED AT ALL. AND SO IT'S JUST A MATTER OF TAKING, AS I UNDERSTAND IT NOW, AND I THINK THE EXPERIMENT SEEMS TO BE -- THE CONCLUSION IS PRETTY CLEAR, THAT BY OPENING IT BACK UP TO CALIFORNIA, WE HAVE MORE QUALITY APPLICANTS TO FUND WITHOUT DISADVANTAGING ON THE RESULTS ANY CALIFORNIA APPLICANT.

CHAIRMAN THOMAS: DIANE.

MS. WINOKUR: OVER A PERIOD OF SEVERAL YEARS NOW, THERE'S BEEN AN ILLUMINATING CHANGE IN THE WAY RESEARCHERS OPERATE. WHEN I FIRST GOT INVOLVED IN THIS, MOST OF THE RESEARCH WAS DONE IN ONE LAB WITH ONE GROUP OF PEOPLE AND THEY WERE VERY CAREFUL NOT TO LET THEIR NEIGHBORS IN THE NEXT LAB KNOW WHAT THEY WERE DOING. THAT DESCRIBES IT. AND THAT'S NO LONGER THE CASE. COLLABORATION IS THE NAME OF THE GAME. AND MOST RESEARCH, WHATEVER IT'S IN OR WHEREVER IT'S BEING DONE, IS DONE COLLABORATIVELY. AND COLLABORATIVELY IN OUR DAY AND AGE MEANS ACROSS STATE LINES, ACROSS COUNTRY LINES, CITY LINES. IT'S JUST THE REALITY.

CHAIRMAN THOMAS: DR. HIGGINS.

DR. HIGGINS: MAYBE MORE CRUDELY I'D JUST LIKE TO EXPAND DR. MILLAN'S AND DR. OLSON'S POINT

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THAT IF WE AS CALIFORNIANS WHO GO OUTSIDE THE STATE WITH OUR CHECKBOOK IN OUR HAND, WE'RE GOING TO GET A BETTER AUDIENCE THAN IF WE'RE JUST GOING OUT AND TRYING TO MAKE FRIENDS. I THINK MONEY CAN BE USED HERE AS A TOOL TO HELP GAIN THE RESOURCES AND THE THINGS THAT WE NEED THAT WOULD COME FROM OUT OF STATE. SO I WOULD SUPPORT THIS SELFISHLY BECAUSE IT'S GIVING US MORE LEVERAGE TO BRING TECHNOLOGY AND RESOURCES IN THE STATE.

CHAIRMAN THOMAS: DR. PRIETO.

DR. PRIETO: I DO UNDERSTAND AND APPRECIATE THOSE ARGUMENTS, BUT I'M CONCERNED ABOUT THE OPTICS OF THIS AND THE ASSURANCES THAT WE GAVE TO THE PEOPLE OF CALIFORNIA WHEN THIS INITIATIVE WAS PASSED. I'M NOT SURE HOW I'M GOING TO VOTE ON THIS MOTION YET, BUT IT DOES RAISE SOME CONCERNS.

CALIFORNIA IS A BIG PLACE WITH A BIG ECONOMY AND VERY ROBUST RESEARCH INFRASTRUCTURE, RESEARCH AND CLINICAL AND BASICALLY ANYTHING YOU CAN NAME, WHICH WE CONTRIBUTE SIGNIFICANTLY TO. I DON'T WANT TO LOSE SIGHT OF THAT.

SUPERVISOR SHEEHY: YES. SO, ONCE AGAIN, I COME TO THE REALITY THAT WE'RE FACING LIMITED RESOURCES. AND SO THERE'S AGREEMENT WHICH WE CAN CONCENTRATE IN CALIFORNIA AND TO MAINTAIN, TO EXPAND

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OUR INTELLECTUAL INFRASTRUCTURE, I THINK, THE MORE IMPORTANT THAT IS.

BUT THE SECOND THING IS JUST, NOT TO BE CRUDELY POLITICAL, BUT WHEN THERE WAS THE TOBACCO TAX FOR CANCER INITIATIVE A FEW YEARS BACK, ONE OF THE THINGS THAT BROUGHT THAT DOWN AND CAUSED IT TO FAIL WAS THAT THEY WERE FUNDING RESEARCHERS OUTSIDE OF CALIFORNIA. I STILL REMEMBER THE TV AD THAT RAN. SO I JUST -- PEOPLE EXPECTED PROP 71 TO BE FOR CALIFORNIA. I'M IN AGREEMENT WITH. I THINK WE HAVE --

CHAIRMAN THOMAS: I'M SORRY. WE'RE LOSING YOU THERE.

DR. DIXON: I SAID I'D QUOTE THE PREVIOUS SPEAKER. I THINK YOU MADE A PROMISE TO THE PEOPLE IN THE STATE, AND FLIPPING BACK AND FORTH, I CANNOT HELP BUT THINK WILL SIMPLY MUDDY THE WATER HERE. AND WE SPENT A GOOD PART OF THE MORNING BASICALLY DECIDING IF WE WERE GOING TO MOVE ONE GRANT CATEGORY TO A FUNDED CATEGORY. IF YOU LOOK AT THE DIFFERENCE BETWEEN THE SCORES OF THOSE, THEY WERE 81, 82, 83, 84. IT WAS NOT THAT THERE WERE THREE TERRIFIC GRANTS AND WE COULDN'T -- AND WE COULD ONLY FUND THREE. MANY OF THOSE GRANTS WERE SEPARATED FROM EACH OTHER BY SIMPLY ONE OR TWO POINTS.

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CHAIRMAN THOMAS: DR. STEWARD.

DR. STEWARD: I DON'T MEAN TO PROLONG THE DISCUSSION. I THINK WE PROBABLY OUGHT TO VOTE ON THIS, BUT JUST TO SAY I WILL PROLONG IT. MARIA'S GOING COME ON, GET GOING HERE. BUT IN THE SAME REQUESTED ACTION, WE JUST REDUCED THE PERCENT OF FUNDING TO AROUND 5 PERCENT; WHEREAS, NIH IS FUNDING SOMEWHERE ABOVE 10 PERCENT. AND NOW WE'RE GOING TO BE SENDING MONEY OUT OF STATE. THE OPTICS ARE THAT WE'RE FUNDING PEOPLE OUTSIDE OF CALIFORNIA. I JUST THINK THOSE ARE TWO IMPOSSIBLE MESSAGES FROM THE POINT OF VIEW OF OPTICS RIGHT NOW. SO I WILL VOTE AGAINST IT. I HAVE BEEN GOING BACK AND FORTH. AND, MARIA, I HEAR YOU AND, PAT, I HEAR YOU, BUT THAT'S THE WAY I'M GOING TO VOTE.

CHAIRMAN THOMAS: DR. MALKAS, DID YOU HAVE ANOTHER COMMENT?

DR. MALKAS: ACTUALLY IT WAS THE POINT ABOUT STANDARDIZATION OF THE STEM CELL PREPS AND THINGS LIKE THAT. WHY DON'T YOU JUST DO IT WITHIN THE STATE? SO WE HAVE MANY INSTITUTIONS. AND I THINK IF WE WERE ACTUALLY ABLE TO STANDARDIZE THE PREPS ACROSS OUR STATE, THAT BECOMES AN INCREDIBLE MILE FOR THE REST OF THE COUNTRY. BUT I LOVE YOU.

DR. MILLAN: THANK YOU, DR. MALKAS. AND

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WE AGREE, AND THAT IS SOMETHING THAT WE ARE PROMOTING.

DR. BERGLUND: I APPRECIATE ALL THE POINTS RAISED, AND I CAN SEE THE VALUE OF CIRM BEING A NATIONAL BRAND. AND I CAN SEE THE VALUE OF THAT MIGHT ACTUALLY HELP OUR RESEARCHERS HERE GET FUNDING OUTSIDE. WHAT I'M WONDERING IS, IN A SITUATION LIKE THIS, AND I APOLOGIZE THAT I DON'T KNOW THE RULES, IS THERE A DEMAND FOR MATCHING FUNDS FROM OUTSIDE CALIFORNIA THAT ACTUALLY COVERS PERSONNEL AND ALL THESE COSTS OUTSIDE, AND OTHERWISE IT WOULDN'T BE FUNDED SO, IN EFFECT, IT DRAWS MONEY OUTSIDE INTO THE PROJECT?

DR. OLSON: SO FOR FOR-PROFIT ENTITIES THAT WOULD APPLY IN CALIFORNIA OR EX CALIFORNIA, THERE'S A 20-PERCENT CO-FUNDING REQUIREMENT.

DR. BERGLUND: IF YOU HAVE AN ACADEMIC PARTNER, YOU COULD REQUEST THEY MIGHT HAVE TO RAISE MONEY ON THEIR END AS WELL.

DR. OLSON: WELL, AS I SAY, THE APPLICANT IS REQUIRED TO MEET THE CO-FUNDING REQUIREMENT. IF THEY HAVE AN ACADEMIC PARTNER WITHIN CALIFORNIA, NO, THERE'S NO REQUIREMENT THAT THE ACADEMIC PARTNER HAVE. IT'S THE APPLICANT THAT THE CO-FUNDING REQUIREMENT FALLS ON AND A FOR-PROFIT APPLICANT.

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CHAIRMAN THOMAS: ARE THERE ADDITIONAL COMMENTS FROM MEMBERS ON THE PHONE? I THINK WE'RE GOING TO NEED A ROLL CALL ON THIS ONE. MR. TOCHER, PLEASE RESTATE THE MOTION.

MR. TOCHER: THE MOTION IS TO APPROVE THE PROPOSED TRAN CONCEPT PLAN AMENDMENT, WHICH IS TO ALLOW OUT-OF-STATE APPLICANTS WITH THE ASSOCIATED ALLOWABLE COSTS.

CHAIRMAN THOMAS: PLEASE CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: NO.

MS. BONNEVILLE: LARS BERGLUND.

DR. BERGLUND: I WOULD ABSTAIN.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: NO.

MS. BONNEVILLE: DEBORAH DEAS. JACK DIXON.

DR. DIXON: NO.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: HOWARD FEDEROFF. JUDY GASSON.

DR. GASSON: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

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MS. BONNEVILLE: STEPHEN JUELSGAARD.
DR. JUELSGAARD: YES.
MS. BONNEVILLE: SHERRY LANSING. BERT LUBIN.
DR. LUBIN: YES.
MS. BONNEVILLE: LINDA MALKAS.
DR. MALKAS: NO.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: SHLOMO MELMED.
DR. MELMED: NO.
MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: NO.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: NO.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: NO.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: NO.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: NO.
MS. BONNEVILLE: OSWALD STEWARD.

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DR. STEWARD: NO.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: NO.
MS. BONNEVILLE: KRISTINA VUORI.
DR. VUORI: NO.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: NO.
MS. BONNEVILLE: IT FAILS 7 TO 15 AND 1 ABSTENTION.
CHAIRMAN THOMAS: THANK YOU, EVERYBODY.
WE ARE GOING TO REQUEST THAT WE GO, THAT LUNCH IS OUT THERE, AND IF EVERYBODY COULD GO AND GET THEIR LUNCH AND GET BACK AT YOUR EARLIEST CONVENIENCE. FIFTEEN MINUTES.
MS. BONNEVILLE: FIFTEEN MINUTES.
CHAIRMAN THOMAS: MARIA SAYS 15 MINUTES, BUT, PLEASE, WE DO HAVE A BIT OF A TIME CONSTRAINT ON THE NEXT ISSUE. BUT IF YOU CAN COME BACK AND WE'LL RESUME IN 15. THANK YOU.
(A RECESS WAS TAKEN.)
CHAIRMAN THOMAS: COULD EVERYBODY COME TAKE YOUR SEATS. WE ARE GOING NOW TO RESUME. WE'RE GOING TO PROCEED TO ITEM NO. 6, DISCUSSION OF

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TRANSITION AND SCIENCE SUBCOMMITTEE MEETING AND POSSIBLE ACTION REGARDING SUSTAINABILITY STRATEGY. SO FOR THOSE ON THE PHONE, WE'RE ON THE LINK TO MY PRESENTATION. SO, AMY, COULD YOU PLEASE, NEXT SLIDE.

SO THE PURPOSE OF THIS DISCUSSION IS TO, AS YOU HAVE HEARD IN DR. MILLAN'S PRESENTATION, THERE'S EVERY LIKELIHOOD THAT WE WILL BE RUNNING OUT OF FUNDS PRIOR TO NOVEMBER OF 2020 OR TO THE YEAR 2020, WHICH WE HAD ORIGINALLY ANTICIPATED AS THE DATE THAT WOULD HAPPEN. SO YOU GO TO NEXT SLIDE PLEASE.

WE WANTED TO START THIS YEAR IN ADDRESSING THE NOTION OF WHAT CAN WE DO TO SUSTAIN CIRM AND ITS WORLD-CLASS PORTFOLIO OF PROJECTS. AND TOWARDS THAT END, IN THE JUNE ICOC MEETING, I CALLED FOR THE ESTABLISHMENT OF A TRANSITION SUBCOMMITTEE TO ADDRESS ISSUES DEALING WITH THE TRANSITION AND SUSTAINABILITY. THE FIRST MEETING OF THAT SUBCOMMITTEE OCCURRED IN SEPTEMBER. AND AT THAT MEETING WE WENT THROUGH A NUMBER OF DIFFERENT IDEAS THAT WERE PUT ON THE TABLE TO ADDRESS THE SUSTAINABILITY QUESTION. HAD A ROBUST DISCUSSION WITH PROS AND CONS AIRED ON EACH AND PRESENTED A REVIEW OF THAT AT THE SEPTEMBER ICOC MEETING, AT

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WHICH POINT WE NOTED THAT, IN THE TRANSITION SUBCOMMITTEE ITSELF, WE HAD DEALT SOLELY WITH THE SUSTAINABILITY ISSUE. WE DID NOT AT THAT MEETING ADDRESS THE ISSUE OF HOW WE WOULD SPEND DOWN OUR REMAINING FUNDS TO GET US TO THAT POINT.

AND BECAUSE THAT TOPIC ENCROACHED ON THE TERRAIN OF THE SCIENCE SUBCOMMITTEE, THE NEXT MOVE WAS TO CONVENE A JOINT SUBCOMMITTEE MEETING OF THE SCIENCE AND TRANSITION SUBCOMMITTEES, WHICH MEETING TOOK PLACE ON NOVEMBER 27TH HERE AT CIRM'S OFFICES. AND AT THAT MEETING, AMONG OTHER THINGS, HAVING HAD THE FIRST MEETING OF THE TRANSITION SUBCOMMITTEE, WE DISTILLED DOWN THE COMMENTS AND IDEAS AND THOUGHTS OF THAT MEETING AND INCORPORATED THEM INTO THE AGENDA OF THE NOVEMBER JOINT SUBCOMMITTEE MEETING. AND IT IS ON THAT JOINT SUBCOMMITTEE MEETING THAT I'M GOING TO REPORT NOW.

WE HAVE UP THERE, JUST SO YOU COULD SEE, WHAT THE AGENDA WAS FOR THAT. WE WENT THROUGH THE DISCUSSION OF SOME OF THE THINGS WE TALKED ABOUT IN THE SEPTEMBER MEETING, AND WE HAD IDENTIFIED THAT, IN ORDER TO GIVE OURSELVES THE BEST POSSIBLE CHANCE AT GETTING ONGOING FUNDING ONCE CIRM HAD RUN OUT OF FUNDS, THERE WAS A TWOFOLD STRATEGY. NO. 1 WAS TO CONTEMPLATE A CITIZEN-LED BOND MEASURE IN NOVEMBER

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OF 2020. AND IF, IN FACT, WE WERE TO RUN OUT OF FUNDS, AS WE HAVE OUTLINED HERE IN DR. MILLAN'S PRESENTATION, IN ADVANCE OF THAT TO PUT TOGETHER A BRIDGE FUNDING FUND-RAISING EFFORT WITH SELECT PHILANTHROPISTS TO GET US THROUGH THAT PERIOD THAT WOULD GET US TO THE NOVEMBER 2020 ELECTION.

WE THEN WENT AND HAD -- DR. MILLAN GAVE THE PRESENTATION, WHICH SHE LARGELY DUPLICATED HERE TODAY, TALKING ABOUT HOW WE WOULD SPEND DOWN THE REMAINING FUNDS, INCORPORATING THE CAP CONCEPT, AND THE PROPOSED BUDGET GOING FORWARD.

AND THEN I TALKED WITH BOB ABOUT THIS NOTION OF THE BRIDGE FUNDING IDEA TO TIDE US OVER TO NOVEMBER 2020, AND THEN WE TALKED ABOUT SOME OTHER FUND-RAISING IDEAS THAT HAD THE MOST POTENTIAL BASED ON WHAT WE HAD TALKED ABOUT AT THE SEPTEMBER MEETING.

NEXT SLIDE PLEASE. SO THE OPTIONS THAT CAME OUT OF THE JOINT SUBCOMMITTEE MEETING THAT WE HAVE DECIDED TO FOCUS ON ARE, NO. 1, A CITIZEN-LED BOND MEASURE IN 2020. WE ALSO HAD AS A BACKUP, WHICH WAS VERY ELEGANTLY DISCUSSED PREVIOUSLY BY SENATOR TORRES, GOING TO THE LEGISLATURE AND PURSUING THE IDEA THAT THEY WOULD PUT SUCH A BALLOT MEASURE ON THE BALLOT. AS YOU KNOW, THERE ARE TWO

WAYS. YOU CAN QUALIFY THROUGH SIGNATURES, WHICH IS WHAT WAS DONE WHEN BOB RAN PROP 71, AND/OR YOU CAN HAVE THE LEGISLATURE PUT THE BALLOT MEASURE ON THERE. THERE ARE MANY PROS AND CONS ATTACHED TO EACH, BUT WE FEEL THAT THE BEST MEASURE, BEST WAY TO GO HERE IS THE CITIZEN-LED MEASURE WITH THE LEGISLATIVE OPTION IN OUR BACK POCKET IN THE EVENT THAT WE NEED, FOR WHATEVER REASON, TO PURSUE THAT.

NEXT SLIDE PLEASE. OKAY. SO I THINK, WITHOUT FURTHER ADO, AS WE DID AT THE JOINT SUBCOMMITTEE MEETING IN NOVEMBER, BOB KLEIN IS HERE TO TALK TO US CONCEPTUALLY ABOUT A CITIZEN-LED BOND MEASURE AND WHAT THAT WOULD ENTAIL. SO VERY HAPPY TO HAVE BOB AND MARY -- IS YIMY HERE TOO? HI, YIMY -- ALL OF WHICH ARE HERE TOO, WITH AMERICANS FOR CURES AS IS OR WAS DON REED. I'M NOT SURE IF DON IS STILL HERE. THERE HE IS. YOU'RE BEHIND THE PODIUM.

THANK YOU ALL VERY MUCH FOR COMING, ALL YOUR TREMENDOUS WORK IN EDUCATING THE PUBLIC THROUGH AMERICANS FOR CURES ON WHAT HAS HAPPENED WITH CIRM AND HOW IT IS PROGRESSING AND HOW OUR PROJECTS ARE PROGRESSING. AND WE GREATLY APPRECIATE ALL OF THAT VERY HARD WORK.

AND NOW, BOB, WOULD INVITE YOU TO SPEAK TO

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US ABOUT THOUGHTS ON A CITIZEN-LED BOND MEASURE IN NOVEMBER OF 2020.

MR. KLEIN: THANK YOU, MR. CHAIRMAN. IT'S ALWAYS A GREAT PRIVILEGE TO ADDRESS THIS BOARD. IT IS EXTRAORDINARY THE WORK THAT YOU'VE DONE IN CARRYING FORWARD THE VISION OF PROPOSITION 71. AND IN THANKING THE BOARD, OF COURSE, I HAVE TO THANK THE DEDICATED STAFF THAT HAS MADE REMARKABLE PROGRESS WITH THE GRANT PROGRAM, BOTH IN VETTING WITH THE PEER REVIEW GROUPS, THE BEST OF THE BEST RESEARCH AND THERAPY PROVISIONS AS WELL, AND ANALYZING AND STRATEGICALLY RECOMMENDING THE BUDGET AND MOVING THIS VERY BROAD PROGRAM, WHICH INCLUDES THE ALPHA CLINICS AND THE BRIDGES PROGRAM, FORWARD IN A REMARKABLE WAY.

THE PATIENTS WHO HAVE COURAGEOUSLY COMMITTED THEMSELVES TO THE HUMAN TRIALS ARE A PART OF THIS INCREDIBLE PROGRESS AS IS THE PUBLIC SUPPORT. AND I WOULD BE REMISS NOT TO THANK THE PAST STAFF BECAUSE IT'S BEEN A CONTINUUM OF REALLY PHENOMENAL PEOPLE. AND SO IT'S A PRIVILEGE THAT ARLENE CHIU IS HERE TODAY AS OUR FIRST SCIENTIFIC DIRECTOR. SHE WAS AT A REMARKABLE STAGE OF PUTTING TOGETHER THESE INITIAL PROGRAMS.

IT IS, IN FACT, EXTRAORDINARY IF YOU LOOK

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AT THE NUMBERS AND STATISTICS, BUT WHAT IS SO COMPELLING AS A PATIENT ADVOCATE, AS A CALIFORNIAN IS TO KNOW AND HAVE THE PRIVILEGE OF KNOWING SOME OF THE PATIENTS THAT HAVE BEEN THROUGH THESE PROGRAMS. EVANGELINA, WHOSE BANNER IS ON THE WALL, WHICH SAYS CURED WHO HAD SCID, IS A YOUNG GIRL WHO MOVED FROM A TOTALLY PROTECTED, ISOLATED, INSULATED ENVIRONMENT WITH VERY LITTLE HUMAN INTERACTION TO BEING ABLE TO SURF ON THE BEACHES OF SOUTHERN CALIFORNIA. THAT IS A JOURNEY THAT IS TRULY REMARKABLE. IT IS A REWARD TO ALL OF US AND CERTAINLY TO THE BOARD, THE STAFF, AND THE PATIENT ADVOCATES WHO HAVE PARTICIPATED IN THAT JOURNEY.

CHRIS BOISSON, THE QUADRIPLEGIC; JAKE JAVIER, WHOSE BANNER IS ON THE WALL, WHO, LIKE CHRIS BOISSON, HAS REGAINED HIS UPPER BODY MOVEMENT AND SOME OF HIS STRENGTH SO HE COULD REALLY PARTICIPATE IN SOCIETY AND COME FROM BEING A QUADRIPLEGIC ON A VENTILATOR TO PARTICIPATING IN THE LIFE OF HIS FAMILY IS A REMARKABLE STORY.

AND SO TAKING ALL OF THESE STORIES TOGETHER WITH THE DOCTORS AND SCIENTISTS WHO HAVE DEDICATED THEIR LIVES TO MAKING THIS POSSIBLE, THIS IS THE CONTEXT FOR WHICH I'M GOING TO DISCUSS THE MORAL IMPERATIVE, I THINK, OF HAVING THE BEST

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POSSIBLE OPTION FOR CONTINUING THIS GREAT EXPERIMENT, THIS VISION THAT THE PEOPLE OF CALIFORNIA ENDORSED, CAME OUT TO VOTE, AND PASSED EVEN THOUGH IT WAS NO. 71 ON THE BALLOT. THAT'S BELOW THE FEDERAL ELECTION OFFICERS, IT'S BELOW THE STATE, IT'S BELOW THE LOCAL ELECTION, IT'S BELOW THE LOCAL BALLOT MEASURES. IT'S AT THE BOTTOM. AND EVEN THOUGH IT WAS AT THE BOTTOM OF THAT LIST OF VOTING OPTIONS, THE U.S. SENATOR AT THE TOP OF THAT BALLOT IN 2004 GOT THE MOST VOTES OF ANY U.S. SENATOR IN ANY ELECTION IN THE HISTORY OF CALIFORNIA, AND PROPOSITION 71 AT THE BOTTOM BECAUSE THE PEOPLE OF CALIFORNIA GOT JUST AS MANY VOTES, SETTING A RECORD FOR THE NATION FOR ANY INITIATIVE.

IT IS THAT GREAT LEGACY THAT YOU'VE UTTERED IN ALL OF YOUR SERVICE; BUT WE NOW HAVE A VERY SPECIAL CHALLENGE IN GOING FORWARD BECAUSE, IN THE LAST 20 YEARS, THE SCIENTIFIC JOURNALISTS WHO CONTRIBUTED SO MUCH IN 2004 ARE LARGELY GONE IN THE PUBLIC MEDIA. NINETY PERCENT OF THE SCIENTISTS IN PUBLIC MEDIA, IN NEWSPAPERS, IN RADIO AND TELEVISION HAVE BEEN REPLACED WITH SPORTS WRITERS OR JUST TOTALLY CUT WITHOUT REPLACEMENT.

THE MEDIA IS VERY DIFFERENT. IT OFFERS OPPORTUNITIES IN SOCIAL MEDIA, AND IT OFFERS

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CHALLENGES BECAUSE WE HAVE TO MAKE CERTAIN THAT WE ARE INFORMING THE PUBLIC NOW, NOT IN 2019 OR 2020, BUT NOW SO THE PUBLIC HAS A STREAM OF VALIDATED MESSAGES ASSOCIATED WITH THE GREAT EDUCATIONAL INSTITUTIONS AND SCIENTIFIC CENTERS OF CALIFORNIA, SO WHEN THEY GET TO THE POINT OF VOTING, THEY CAN DISTINGUISH TRUE SCIENTIFIC NEWS AND ACHIEVEMENTS FROM FICTIONAL ATTACKS, WHICH WILL BE PLENTY.

WE NEED TO KNOW THAT THIS GREAT REVOLUTION IS EMBRACED AGAIN BY THE CIVIC ORGANIZATIONS, STARTING WITH THE PATIENT ADVOCATES, BUT INCLUDING THE STATE CHAMBERS OF COMMERCE, WHICH ENDORSED THIS INITIATIVE, FROM SAN DIEGO TO SAN FRANCISCO, INCLUDING THE ORANGE COUNTY BUSINESS COUNCIL. AND STATE CHAMBER DOESN'T HAVE A HISTORY OF ENDORSING A GREAT NUMBER OF ISSUES RELATED TO BONDS AND TAXES, BUT THEY DID STEP UP TO THE PLATE, UNDERSTANDING THAT THIS IS REALLY THE FUTURE -- THIS IS CALIFORNIA'S CONTRIBUTION TO THE FUTURE OF MEDICINE.

FROM WHERE I STAND IN UNDERSTANDING THAT IT'S ONLY 2007 WHERE IN MAY WE GOT OUT OF THE STATE SUPREME COURT AND COULD BEGIN OUR MAJOR FUNDING, WE ARE AT TEN YEARS. AND I CAN TELL YOU THAT WHETHER IT'S WHAT WE PUT INTO THE LEGISLATIVE ANALYSTS IN 2003 OR WHAT THE REAL ADS WERE AS VERSUS THE

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HYPOTHETICAL ADS PEOPLE HAVE TALKED ABOUT, THIS IS FAR BEYOND THE ACHIEVEMENTS WE EXPECTED IN THIS TIME FRAME. TO HAVE 43 HUMAN TRIALS FUNDED BY THIS AGENCY, TO HAVE ANOTHER 14 HUMAN TRIALS WHERE THE ORIGINAL WORK WAS FUNDED BY THIS AGENCY BUT OTHERS ARE NOW FUNDING IT IS AN UNBELIEVABLE LEVEL OF SUCCESS. AND IF YOU LOOK AT THE OBJECTIVES THE BOARD ADOPTED IN THE ANCIENT DAYS WHEN OSWALD STEWARD AND FRANCISCO PRIETO AND JEFF SHEEHY WERE ON THE BOARD, THIS BLOWS PAST THOSE GOALS AT AN EXTRAORDINARY LEVEL.

AND THERE ARE THOSE WHO ASK, WELL, THIS MAY BE GREAT ACHIEVEMENTS. WELL, WILL THE PUBLIC STAND AGAIN BEHIND THIS VISION? THIS TIME WE DON'T HAVE JUST A VISION. I MEAN WE HAVE A SURFER. WE HAVE PATIENTS WHO HAVE HAD THEIR LIFE RESTORED, WHETHER IT'S CHRIS BOISSON OR JAKE OR CANCER PATIENTS.

(THE PHONE TRANSMISSION WAS DROPPED AND THEN CONTINUED AS FOLLOWS:)

-- WHAT THE VALUE IS FOR THE SOCIETY. THIS IS THE BRIDGE TO THE FUTURE OF HEALTHCARE. THIS IS CALIFORNIA'S CONTRIBUTION. ONE SPAN OF ONE BRIDGE VERSUS A BRIDGE TO THE FUTURE OF HEALTHCARE AS A CONTRIBUTION FROM CALIFORNIA SCIENTISTS,

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DOCTORS, PATIENT ADVOCATES, AND CIVIC SOCIETY.

IF WE LOOK BACK TO THE HISTORY OF HOW WE ACCOMPLISHED THE FIRST INITIATIVE, WE MEANING A BROAD GROUP OF CITIZENS ADVISORY GROUP, A PATIENT ADVOCACY ADVISORY COMMITTEE, A SCIENTIFIC ADVISORY COMMITTEE, AND A GREAT NUMBER OF DEDICATED DONORS, WHO WE DEEPLY APPRECIATE, AND WE LOOK FORWARD, WE CAN EXPECT THAT THE 7 MILLION VOTES, THE 7 MILLION VOTERS WHO VOTED FOR THIS INITIATIVE, IF THEY HAVE THE INFORMATION ABOUT THE ACHIEVEMENT, GOES FAR BEYOND JUST THE CLINICAL TRIALS WHICH ARE EXTRAORDINARY AND DEEPLY IMPORTANT TO THESE ADVANCES AS A PATH TO SUCCESS, WE HAVE 2600 PEER REVIEWED DISCOVERIES PUBLISHED IN MAJOR SCIENTIFIC LITERATURE. WE HAVE TRANSLATIONAL PIPELINES IN PLACE WHICH HAVE TO BE MAINTAINED. WE HAD 40 NOBEL PRIZE WINNERS WHO SUPPORTED THE ORIGINAL INITIATIVE. I THINK IT IS SAFE TO SAY THAT, LOOKING AT THE ACHIEVEMENTS THAT HAVE OCCURRED, THAT IS AN ELEMENT OF SUPPORT WE CAN COUNT ON GOING FORWARD.

BUT WHAT WE NEED TO UNDERSTAND IS THAT ULTIMATELY IT IS THE VOTERS OF CALIFORNIA THAT WE HAVE TO INFORM IN A REPORT BACK TO THE PUBLIC TO HONOR OUR OBLIGATION TO THEM BECAUSE THEY PUT FAITH IN THIS VISION. AND THROUGH AMERICANS FOR CURES,

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FOR WHICH I SERVE AS CHAIRMAN, WE ARE ATTEMPTING TO WORK WITH THE ACADEMIC INSTITUTIONS, WITH THE RESEARCH INSTITUTIONS, INDEPENDENT RESEARCH INSTITUTIONS OF THE STATE TO PROVIDE AN INFORMATIONAL MARRIAGE OF THE PATIENT ADVOCATES MESSAGING AND THE SCIENTIFIC MESSAGING SO THE PUBLIC IS REALLY FULLY INFORMED.

TODAY WE KNOW THAT THAT SUPPORT FROM THE PUBLIC IS IN THE RANGE OF 70 PERCENT EVEN IN POLLING. EVEN AFTER A NEGATIVE MESSAGE HAS REBUTTED THE POSITIVE MESSAGE, THE VOTERS HOLD AT ABOUT A 70-PERCENT APPROVAL. FOR THAT TO CONTINUE, IN THE FACE OF WHAT WE CAN EXPECT WILL BE A LOT OF DISINFORMATION, WE HAVE A LOT OF WORK TO DO IN COMMUNICATING WITH THE VOTERS WITH INDIVIDUAL PATIENT STORIES, WITH SCIENTIFICALLY VETTED ARTICLES, WITH SPOKESMEN FROM SCIENCE SIDE BY SIDE WITH PATIENT ADVOCATES.

AND WHEN YOU LOOK TO THE VOTERS, YOU ASK WHY 2020? WHY NOT 2018? WE HAVE TREMENDOUS PROGRESS. WE HAVE A GREAT STORY TODAY. WELL, FIRST OF ALL, THE POLLS ARE NOT ALWAYS RIGHT, AS WE CAN GUESS FROM THE LAST ELECTION IN THIS COUNTRY FOR THE PRESIDENCY. SECONDLY, IF YOU BUILD A VERY STRONG FOUNDATION OF DEEP INFORMATION THAT IS VALIDATED BY

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THE INSTITUTIONS WITHIN EACH REGION, YOUR ABILITY TO PREDICT TURNOUT, JUST AS WE DID IN 2004, IS MUCH HIGHER.

IF WE LOOK BACK AT THE NUMBERS, IN 2004 THERE WERE 7 MILLION VOTES FOR PROPOSITION 71, AS I STATED EARLIER, OUT OF A TOTAL OF 12.5 MILLION VOTES. IF YOU LOOK AT WHAT HAS HAPPENED IN THE OFF-YEAR ELECTIONS IN CALIFORNIA, AFTER THE INSTITUTION IN PARTICULAR OF THE RULE THAT THE TOP TWO CANDIDATES, EVEN THOUGH THEY MIGHT BE THE SAME PARTY, WILL STAND FOR ELECTION FOR STATEWIDE OFFICE, YOU SEE A HUGE DROP-OFF IN TURNOUT IN OFF-YEAR ELECTIONS. IN 2014 JERRY BROWN, A POPULAR GOVERNOR, WAS REELECTED WITH 4,000,380 VOTES OUT OF A TOTAL VOTE TURNOUT OF 7 MILLION. THINK ABOUT THAT. A DECADE AFTER PROPOSITION 71, THE STATE HAS GROWN, AND YET, INSTEAD OF 12 MILLION VOTES TURNING OUT, THERE'S 7 MILLION VOTES THAT TURN OUT. WELL, WHAT HAPPENS WHEN YOU HAVE A LOW TURNOUT? YOU SKEW TOWARDS VERY CONSERVATIVE VOTERS WHO ARE NOT AS VISIONARY, WHO ARE NOT AS PROGRESSIVE, AND WE NEED TO UNDERSTAND THAT. JUST AS WE GAVE THE ENTIRE STATE THE BEST OPPORTUNITY TO TURN OUT AND VOTE IN 2004, WE NEED TO DO THAT AGAIN IN 2020.

AGAIN, BY COMPARISON, EVEN THOUGH IN 2014

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THE TURNOUT WAS 7.3 MILLION, IN A PRESIDENTIAL ELECTION IN 2016, THE TURNOUT WAS 14.2 MILLION. SO THE PATTERN IS --

(THE PHONE TRANSMISSION WAS INTERRUPTED. AFTER IT WAS REINSTATED, MR. KLEIN'S PRESENTATION CONTINUED AS FOLLOWS:)

MR. KLEIN: ...FOR PATIENTS. THEY HAVE SCIENTIFIC ADVISORY BOARDS, THEY HAVE ATTORNEYS. WHAT REALLY IS HAPPENING HERE IS THAT THE ORGANIZATIONS THAT REPRESENT PATIENTS, WHICH ARE A SURROGATE FOR THE 45 PERCENT OF THE PUBLIC THAT AT ANY ONE TIME HAS A FAMILY MEMBER, A BROTHER, A SISTER, A GRANDPARENT, A CHILD WHO HAS A CHRONIC DISEASE, THIS IS THEIR SURROGATE. THEY LOOK AT THESE ORGANIZATIONS AND THINK, LOOK, ONE OR TWO OF THEM CAN BE WRONG. SEVENTY, 80 OF THEM, COULD THEY REALLY BE WRONG? THE ODDS ARE NOT HIGH. THIS IS A TRUSTED GROUP THAT REPRESENTS THE PUBLIC.

SO WE HAVE AN ADVANTAGE THAT IF WE HAVE HIGH TURNOUT WITH A PUBLICLY ADVANCED INITIATIVE, WITH A PRIVILEGED MESSENGER, BECAUSE PATIENT ADVOCATES BEING A TRUSTED SURROGATE ARE PRIVILEGED MESSENGERS, AND WE HAVE A PRIVILEGED MESSAGE. THIS IS ABOUT THE HEALTH AND FUTURE HEALTH OPPORTUNITY OF SOMEONE THEY LOVE. THAT IS A MESSAGE THAT

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PENETRATES A LOT OF NOISE. AND IF YOU NEED TO COMMUNICATE THROUGH SOCIAL MEDIA, YOU BETTER BE ABLE TO PENETRATE THROUGH A LOT OF NOISE.

THAT'S PARTICULARLY TRUE IF YOU DON'T HAVE A LOT OF PUBLIC MEDIA WITH GREAT SCIENCE JOURNALISTS IN PLACE. AND WE DEEPLY APPRECIATE THOSE PUBLIC SCIENCE MEDIA JOURNALISTS WHO ARE IN PLACE.

SO IF YOU LOOK AT WHERE WE ARE TEN YEARS INTO THIS PROCESS AND REALIZE THAT IN 2004 WE SAID IT WOULD BE 14 YEARS BEFORE WE HAVE THE FIRST THERAPY THAT WOULD BE AVAILABLE TO THE PUBLIC, THAT'S FOUR YEARS FROM NOW, AND WE HAVE THERAPIES NOW GETTING TO THE POINT THEY'RE AVAILABLE TO THE PUBLIC. IT'S JUST THE BREAKING EDGE, BUT IN THE NEXT THREE YEARS WE SHOULD STRATEGICALLY HAVE, THROUGH THE HUMAN TRIALS THAT ARE GOING ON NOW, OTHERS GETTING TO THAT POINT. SO WE WILL HAVE MORE VISIBLE, SPECIFIC BENEFITS TO PATIENTS. IT IS IMPORTANT TO REALIZE THAT THE HUMAN TRIALS ARE VERY STRONG MILESTONES THAT THE PUBLIC CAN CREDIBLY BELIEVE BECAUSE THE BREADTH OF THOSE TRIALS WILL LEAD TO THERAPIES TO HELP THEIR FAMILIES, THEIR FRIENDS, THEIR LOVED ONES. WE WILL HAVE A DEEPER FOUNDATION IN PROVIDING THAT PROOF TO THE PUBLIC.

IT IS IMPORTANT TO REALIZE AND BE

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REALISTIC ABOUT THE DIFFICULTIES THAT ARE BEFORE US. WE HAVE TO ORGANIZE FOR THESE CHALLENGES, AND WE HAVE TO MAKE CERTAIN THAT THERE IS BRIDGE FUNDING IN PLACE. WHETHER IT'S 222 MILLION OR IT'S 300 MILLION, WE NEED VERY SUBSTANTIAL BRIDGE FUNDING. HOPEFULLY IT'S MORE THAN YOUR MINIMUM THRESHOLD TO CONTINUE THIS PIPELINE BECAUSE, AS WE SAW TODAY, THERE ARE TREMENDOUS OPPORTUNITIES THAT ARE THERE THAT AREN'T AVAILABLE FOR FUNDING BECAUSE OF THE CONSTRAINT. OUR FUNDING LEVEL IS, AS DR. STEWARD HAS SAID, EVEN BELOW NIH FUNDING LEVELS BECAUSE OF THE SCARCITY OF RESOURCES AT THIS POINT.

SO IT IS A PRIVILEGE FOR ME TO BE PART OF THIS EFFORT. AND AS DICKENS, CHARLES DICKENS SAID, "IT IS THE BEST OF TIMES. IT IS THE WORST OF TIMES. IT IS A TIME OF GREAT WISDOM AND GREAT FOOLISHNESS." MAY WE, WITH THE PUBLIC IN CALIFORNIA, REPRESENT WISDOM AS THE BENEFICIARIES OF THE GREAT SCIENTISTS AND DOCTORS OF CALIFORNIA, FOR THE BENEFIT OF OUR PATIENTS AND OUR PUBLIC. IT IS A PRIVILEGE TO HOPEFULLY PARTICIPATE IN THE LEADERSHIP OF A PUBLIC INITIATIVE IN 2020. THE PUBLIC WILL MAKE THE DECISION AT THAT TIME IF THAT'S THE RIGHT OPTION. THANK YOU.

(APPLAUSE.)

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CHAIRMAN THOMAS: THANK YOU VERY MUCH, BOB.

ANY COMMENTS BY MEMBERS OF THE BOARD?

MR. TORRES: I JUST WANT TO THANK YOU, BOB, FOR TAKING UP THE CHALLENGE ONCE AGAIN. IT'S NOT OFTEN THAT WE HAVE SOMEONE WHO IS WILLING TO GO BACK TO THE FIELD AND BACK TO CENTER STAGE AT GREAT COST TO YOU AND TO YOUR FAMILY. BECAUSE OF YOUR COMMITMENT I, FOR ONE, AND I'M SURE ALL THE BOARD, APPRECIATES THE FACT THAT YOU'RE WILLING TO TAKE ON THIS LEADERSHIP AGAIN IN 2020. THANK YOU.

CHAIRMAN THOMAS: HERE. HERE. I THINK THAT ABOUT SUMS UP THE SENTIMENT THAT EVERYBODY HAS HERE. SO THANK YOU. THANK YOU, BOB, VERY MUCH. AND THANK YOU TO THE MEMBERS OF YOUR TEAM FOR ALL THE HARD WORK ON EVERYTHING YOU'RE DOING.

SO, AMY, NEXT SLIDE PLEASE.

SO, AS I MENTIONED, DR. MILLAN HAS IDENTIFIED THAT WE NOW BELIEVE, BASED ON OUR CURRENT SPENDING RATE AND THE QUALITY OF BEST-IN-CLASS PROJECTS IN THE PIPELINE, THAT WE COULD WELL RUN OUT OF RESEARCH DOLLARS BY Q4 2019.

WE HAD A DISCUSSION IN-HOUSE WHICH POSED THE FOLLOWING QUESTION: IF WE WERE TO FUND PROGRAMS IN A WAY THAT WOULD SUSTAIN CIRM'S ACTIVITIES AND

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GET US FROM Q4 OF 2019 TO NOVEMBER OF 2020 IN A WAY THAT MAINTAINS THE MOMENTUM THAT KEEPS THINGS GOING AT A RATE THAT WILL ALLOW US TO ADDRESS VERY SUBSTANTIALLY THE DIFFERENT PRONGS OR PILLARS OF OUR EFFORT, WHAT WOULD THE DOLLAR AMOUNT BE THAT WE MIGHT NEED TO BRIDGE FROM Q4 OF 2019 TO NOVEMBER OF 2020? AND THE FIGURE THAT THE TEAM CAME UP WITH WAS $222 MILLION.

SO THE DISCUSSIONS THEN TURNED TO HOW WOULD WE AMASS 222 MILLION, WHICH IS CERTAINLY A VERY, VERY NONTRIVIAL NUMBER. SO WE'VE HAD DISCUSSIONS PRELIMINARILY ON THE CONCEPT OF RAISING A BRIDGE FINANCING ROUND, BRIDGE FUNDING ROUND, RATHER, TO TALK TO SUPPORTERS OF CIRM FROM THE HIGH NET WORTH COMMUNITY WHO COULD POSSIBLY BE INTERESTED IN PARTICIPATING IN A CONSORTIUM OF BRIDGE FUNDERS TO HELP US MEET OUR $222 MILLION GOAL, WHICH WOULD BE EXTRAORDINARY, AND, IF WE'RE LUCKY, PERHAPS EVEN BEYOND THAT TO ALLOW FOR ENLARGING THE AMOUNT OF FUNDS AVAILABLE FOR EACH OF THE PILLARS.

ON THE SLIDE YOU'VE GOT UP THERE, WE BREAK DOWN THE 222. AND YOU CAN SEE UP THERE HOW THAT BREAKS DOWN AND WHERE THE MONEY WOULD GO: 114 MILLION TO THE CLIN AWARDS, 40 MILLION TO THE TRAN AWARDS, 20 TO THE DISC, 16 TO THE EDUCATION. THAT

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EDUCATION WOULD CONTEMPLATE POTENTIALLY TRAINING AWARDS THAT WE HAVEN'T HAD FOR A COUPLE OF YEARS, BUT ARE THINGS THAT POTENTIAL DONORS FIND VERY INTERESTING AND WORTHY OF SUPPORT.

ON THE INFRASTRUCTURE FRONT, ANOTHER 16 MILLION. WE COULD CONTEMPLATE ADDING TWO MORE ALPHA CLINICS TO WHAT WE HAVE NOW, WHICH, AS YOU KNOW FROM THE SEPTEMBER BOARD MEETING, IS FIVE.

AND THEN LAST, BUT NOT LEAST, THOUGH WE HAVE ADMIN FUNDS THAT TODAY WILL TAKE US THROUGH EARLY 2021, THE CONCEPT WOULD BE TO HAVE 80 PERCENT OF WHATEVER WE RAISE GO TOWARDS ADDITIONAL ADMIN FUNDS JUST TO MAKE SURE WE HAD ENOUGH MONEY THAT IN THE, WE HOPE, UNLIKELY AND CERTAINLY UNHAPPY EVENT THAT THE BOND MEASURE DOESN'T PASS IN 2020, WE WOULD HAVE ENOUGH ADMIN DOLLARS TO COMPETENTLY ADMINISTER THE THEN BALANCE OF THE EXISTING PORTFOLIO AS WE WOULD HAVE TO WIND DOWN CIRM THROUGH THE YEAR 2023, THE LAST AWARDS HAVING BEEN MADE IN 2019. SO THAT IS THE 222.

WE'VE HAD SOME PRELIMINARY CONFIDENTIAL DISCUSSIONS WITH PARTICULAR PARTICIPANTS THAT WE HOPED WOULD JOIN US IN THIS EFFORT. WE THINK THAT THE CHANCE TO STAND ON THE SHOULDERS OF $3 BILLION WORTH OF FUNDING AND A WORLD-CLASS PORTFOLIO THAT IS

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SECOND TO NONE IS A VERY INTERESTING AND POSITIVE OPPORTUNITY. SO GOING FORWARD, WE ARE LOOKING TO MAKE THAT HAPPEN.

AMY, NEXT SLIDE. SO SPECIFICALLY, THE DONATIONS CAN TAKE MANY FORMS. THE BEST FORM WOULD BE UNRESTRICTED, WHICH WOULD ALLOW CIRM TO PUT THE FUNDING INTO THINGS AND PILLARS AS WE SEE FIT, BUT YOU COULD END UP HAVING FUNDING FOR DIFFERENT PROGRAMS. YOU COULD HAVE FUNDING FOR DIFFERENT DISEASES. THERE ARE LOTS OF WAYS TO COBBLE THIS TOGETHER. BUT WE THINK THAT COMBINED THAT WE ARE OPTIMISTIC HERE THAT WE CAN MAKE THIS VERY, VERY CHALLENGING THING HAPPEN.

AS YOU SEE HERE, WE'VE SET AS GOALS FOR THIS BRIDGE FUND-RAISING, THE FIRST 55 MILLION AS OF Q4 OF 2018; THE SECOND -- AND WE HAD TO MAKE THIS ALL ADD UP TO 222, SO THE NUMBER IS SLIGHTLY DIFFERENT FROM GOAL YEAR TO GOAL YEAR -- Q2 2019, AN ADDITIONAL 55.5, ANOTHER 55.4 BY Q4 2019, AND THE BALANCE OF 56 BY Q1 2020. AND IF WE ARE SUCCESSFUL, WE WILL THEN HAVE FULLY FUNDED WHAT WE BELIEVE TO HAVE BEEN A VERY ROBUST ADDITIONAL YEAR OF ALL FIVE OF OUR PROGRAMS PLUS ADDITIONAL ADMINISTRATIVE EXPENSE. AND THEN WHEN WE GET TO Q4 2020, AT WHICH POINT WE WOULD HAVE THE BOND INITIATIVE WHICH WE ALL

ARE VERY OPTIMISTIC AND HOPEFUL, BASED ON A VARIETY OF FACTORS, NOT THE LEAST OF WHICH IS WE HAVE A TREMENDOUS ASSET THAT WE'RE SELLING HERE TO THE PUBLIC ON WHAT WE'VE BEEN ABLE TO ACHIEVE, WE ARE VERY MUCH HOPEFUL THAT THAT WILL BE THE CONCLUSION OF THE ELECTORATE AT THAT POINT AS WELL.

GO TO THE NEXT. SO THE ONE OTHER FUNDING MECHANISM THAT'S GETTING SOME RESONANCE AND SOME EARLY CONFIDENTIAL DISCUSSIONS WITH POTENTIAL FUNDERS IS THE NOTION OF CO-FUNDING PROJECTS EITHER THAT WE HAVE PREVIOUSLY FUNDED BECAUSE THEY'RE IN A SUBJECT MATTER THAT A DONOR FINDS INTERESTING OR IN A SUBJECT MATTER THAT THEY FIND INTERESTING THAT WE MAY BE FUNDING GOING FORWARD. IN ORDER TO IMPLEMENT THIS, THE POTENTIAL DONOR WOULD AGREE TO ABIDE BY THE RECOMMENDATIONS OF THE GWG, WOULD NOT ENTERTAIN THE IDEA OF GOING AND CONDUCTING THEIR OWN INDIVIDUAL REVIEW, AND WOULD JUST PIGGYBACK, AS I SAY ON THE SLIDE HERE, WITH THE GWG AND THE BOARD APPROVAL. SO WE THINK THAT THIS MAY BE SOMETHING THAT PROVIDES POTENTIALLY SOME SIGNIFICANT FUNDING IN ADDITION TO THE BRIDGE IDEA, OR IT COULD BE INCORPORATED INTO IT TO HELP MAKE IT HAPPEN.

SO THOSE ARE THE IDEAS. THIS IS WHAT WE DISCUSSED AT THE TRANSITION SUBCOMMITTEE. WE THINK

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WE HEAR AT CIRM AND BOB AND HIS TEAM AT AMERICANS FOR CURES THINKS THIS IS A VIABLE GAME PLAN THAT WOULD ALLOW US TO SUSTAIN THE AGENCY GOING FORWARD. NOBODY EXPECTS THIS TO BE EASY. EVERY ASPECT OF IT IS GOING TO BE A MAJOR CHALLENGE, BUT WE BELIEVE THIS IS THE BEST WAY TO GO TO SUSTAIN OUR WORK.

SO WITH THAT, ARE THERE ANY QUESTIONS OR COMMENTS?

DR. HIGGINS: TO WHAT EXTENT IS THE BOARD AND THE STAFF PROHIBITED FROM BEING INVOLVED IN THIS?

CHAIRMAN THOMAS: EXCELLENT QUESTION. MR. TOCHER.

MR. TOCHER: UP TO THE POINT WHERE THE BALLOT MEASURE IS ACTUALLY QUALIFIED AND ON THE BALLOT, THERE'S A FAIR AMOUNT OF LATITUDE IN TERMS OF PREPARING COMMENTARY ON IT AND PROVIDING INFORMATION AND WORKING ON CRAFTING LANGUAGE, IF THAT INPUT IS SOUGHT. HOWEVER, ONCE A MEASURE QUALIFIES FOR THE BALLOT, THE BOARD'S ACTIONS ARE RESTRICTED TO TAKING A FORMAL ENDORSEMENT ON THE MEASURE IN A PUBLICLY NOTICED HEARING THAT PROVIDES FOR PUBLIC INPUT, PROVIDING OBJECTIVE ANALYSIS ON THE BOARD WEBSITE, FOR INSTANCE, RESPONDING TO REQUESTS FOR INFORMATION THAT DO NOT TAKE THE FORM

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OF ADVOCATING FOR A MEASURE'S DEFEAT OR PASSAGE.

SO YOUR ACTIVITY MUST BE MUCH MORE CIRCUMSPECT AFTER A MEASURE QUALIFIES FOR THE BALLOT. THAT DOES NOT RESTRICT YOU FROM ANYTHING YOU WOULD DO IN YOUR PERSONAL CAPACITY, OF COURSE. AND WE HAVE HISTORICALLY SENT OUT INFORMATION, MEMORANDA DESCRIBING THIS IN GREATER DETAIL FOR YOU, AND WE WILL DO SO AGAIN.

CHAIRMAN THOMAS: ANY OTHER COMMENTS BY MEMBERS OF THE BOARD EITHER HERE OR ON THE PHONE? OKAY. I DON'T BELIEVE THIS IS ANYTHING THAT REQUIRES A VOTE. THIS IS SORT OF AN INFORMATIONAL ITEM. WE WILL OBVIOUSLY KEEP YOU POSTED AT EVERY STEP OF THE WAY HERE AND LOOK FORWARD TO MAKING THIS HAPPEN. IT BASICALLY HAS TO HAPPEN BECAUSE WE HAVE TOO MANY GOOD THINGS GOING ON HERE TO HAVE IT COME TO A SCREECHING HALT WHEN WE RUN OUT OF FUNDS.

NO OTHER COMMENTS, ANYBODY ON THE PHONE? OKAY. THANK YOU. AND, BOB AND TEAM, THANK YOU. I KNOW YOU HAVE TO RUN. THANKS VERY MUCH FOR BEING HERE FOR THIS MEETING AND THE SUBCOMMITTEE MEETING. AND HAVE A GOOD TRIP BACK TO PALO ALTO.

MR. KLEIN: VERY NICE PRESENTATION.

CHAIRMAN THOMAS: THANK YOU. OKAY. I THINK THIS LOOKS LIKE, SINCE WE'VE BEEN DOING

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VARIOUS THINGS -- OKAY. WE'RE GOING TO DO SOMETHING ELSE OUT OF ORDER. THE ALWAYS POPULAR CONSENT CALENDAR, WHICH HAS A NUMBER OF NONCONTROVERSIAL BUT, NONETHELESS, VERY IMPORTANT POINTS.

I'M SORRY. BEFORE GET TO THE CONSENT CALENDAR, MARY BASS FROM AMERICANS FOR CURES WOULD LIKE TO MAKE A COMMENT.

MS. BASS: THANK YOU. AS CHAIRMAN THOMAS MENTIONED, MY NAME IS MARY BASS. AND I'M THE EXECUTIVE DIRECTOR OF AMERICANS FOR CURES, THE NONPROFIT OF WHICH BOB KLEIN IS CHAIR.

SO FIRST I WANT TO THANK EACH AND EVERY ONE OF YOU FOR BEING HERE AND ALL THE WORK THAT YOU DO, TO DR. MILLAN, TO CHAIRMAN THOMAS, TO SENATOR TORRES, SUPERVISOR SHEEHY, AND ALL OF YOU HERE.

I WANT TO TAKE A MOMENT TO PAY TRIBUTE TO AND RECOGNIZE THE PATIENTS AND THE PATIENT ADVOCATES WHO ARE THE REASON BEHIND WHY WE ALL DO WHAT WE DO. AND THAT INCLUDES BOTH THOSE HERE TODAY AND THOSE WHO ARE NO LONGER WITH US. SO SPECIFICALLY, DAVID AND FRANCES SALDANA WHO TRAGICALLY LOST THEIR SON MICHAEL TO HUNTINGTON'S DISEASE AND CONTINUE TO ADVOCATE, TO FIGHT HUNTINGTON'S. TO ADRIENNE SHAPIRO, WHO IS HERE AS WELL, WHOSE DAUGHTER HAS SICKLE CELL DISEASE. TO DON REED WHOSE SON ROMAN,

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OF COURSE, WAS PARALYZED. TO DIANE WINOKUR, WHOSE SON'S LIFE WAS TRAGICALLY CLAIMED BY ALS. AND TO JENNIFER RAUB, WHO'S NOT HERE TODAY, BUT WHO IS A TIRELESS ADVOCATE FOR PARKINSON'S, AS, OF COURSE, IS DAVID HIGGINS. AND, OF COURSE, TO BOB KLEIN WHOSE SON WAS, IS THE REASON THAT ALL OF THIS EXISTS TODAY.

SO WITH THESE STORIES, WE'RE EXCITED FOR THE JOURNEY THROUGH 2020 TO EDUCATE THE PUBLIC ON THE TREMENDOUS SUCCESSES OF THE CALIFORNIA EXPERIMENT, AT WHICH POINT THE VOTERS WILL MAKE THE CHOICE OF WHETHER TO CONTINUE THEIR INVESTMENT. SO THANK YOU ALL.

(APPLAUSE.)

CHAIRMAN THOMAS: THANK YOU, MARY.

SO DO WE HEAR A MOTION TO APPROVE THE CONSENT ITEMS?

DR. JUELSGAARD: SO MOVE.

CHAIRMAN THOMAS: MOVED BY MR. JUELSGAARD.

DR. GASSON: SECOND.

CHAIRMAN THOMAS: SECONDED BY DR. GASSON. ANY COMMENT ON ANY OF THESE? HEARING NONE, ANY COMMENT BY MEMBERS ON THE PHONE? WE CAN DO THIS ON A VOICE VOTE PLUS ROLL.

MR. TOCHER: PUBLIC COMMENT.

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CHAIRMAN THOMAS: SORRY. PUBLIC COMMENT. I FORGOT. NO PUBLIC COMMENT. MARIA, I WILL FIRST ASK, AND IF YOU CAN POLL THOSE ON THE PHONE. ALL THOSE IN FAVOR OF THIS MOTION IN THE ROOM PLEASE SAY AYE. OPPOSED? ABSTENTIONS? MARIA, PLEASE CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: YES.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: JACK DIXON.

DR. DIXON: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY. KRISTINA VUORI.

DR. VUORI: YES.

MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU. WE'LL NOW PROCEED TO THE CHAIR'S REPORT. SO I HAVE, IN ADDITION TO THE JOINT SUBCOMMITTEE PRESENTATION, I HAVE A NUMBER OF THINGS I WANTED TO BRING TO THE BOARD'S ATTENTION AS I THOUGHT THEY WERE ITEMS OF

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INTEREST. NO. 1, IN OCTOBER, AS WE'VE ALWAYS HAD, THERE WAS THE MEETING ON THE MESA DOWN IN LA JOLLA, WHICH IS AN ANNUAL CONVENING OF BIOTECH COMPANIES IN THE STEM CELL SPACE, INVESTORS, PATIENT ADVOCATES, SOME POLITICAL FOLK, ETC. IT'S ALWAYS ONE OF THE SORT OF BELLWETHER EVENTS AT WHICH YOU CAN GAUGE THE PROGRESS OF THE INDUSTRY.

A NUMBER OF US WERE DOWN THERE FOR THIS. JUST A FEW STATS OF INTEREST. THERE WERE 60 PRESENTING COMPANIES AT THE EVENT PLUS A WHOLE BUNCH OF OTHERS THAT DIDN'T PRESENT. AND OF THOSE, A NUMBER ARE VERY FAMILIAR NAMES: ASTERIAS, CAPRICOR, CELLULAR DYNAMICS THAT WAS INVOLVED WITH IPS CELL BANK, JCYTE, ORCHARD, WHICH IS THE COMPANY THAT DON KOHN FORMED, SANGAMO, AND VIACYTE, AMONGST OTHERS. SO CIRM-FUNDED PROJECTS WERE VERY WELL REPRESENTED. THEY HAD JUST UNDER A THOUSAND ATTENDEES AT THIS YEAR'S MEETING, AND ARE SORT OF WORKING THEIR WAY INTO OUTGROWING THE ESTANCIA HOTEL IN LA JOLLA, WHICH HAS BEEN A GREAT VENUE SINCE ITS INCEPTION.

THE BIG PURPOSE OF THIS EVENT IS TO GET MEETINGS, NETWORKING MEETINGS, POTENTIAL COLLABORATION MEETINGS. THEY SAID THAT THEY HAD 1450 SUCH MEETINGS. I'M NOT QUITE SURE HOW THEY GAUGE THAT, BUT THERE WERE -- I KNOW MARIA AND ABLA

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AND PAT AND OTHER MEMBERS OF THE TEAM HAD A WHOLE BUNCH OF MEETINGS SORT OF DAWN TO DUSK AND MADE SIGNIFICANT PROGRESS IN DISCUSSIONS WITH EITHER EXISTING AWARDEES OR POTENTIAL. SO IT WAS, AS IT TENDS TO BE, A REAL SUCCESS.

SECOND THING I WANTED TO REPORT TO YOU WAS, AS YOU KNOW, ANNUALLY WE REPORT TO THE STATE CONTROLLER, BETTY YEE, GREAT FRIEND OF THE SENATOR'S, WHO CONVENES THE SO-CALLED CFAOC, WHICH IS A COMMISSION THAT MEETS TO HEAR ABOUT CIRM AND THE PROGRESS THAT IT'S MADE, THE BUDGETARY MATTERS, HOW IT'S HANDLING THE DOLLARS IN A WAY THAT IS COMMENSURATE WITH EXPERT STEWARDSHIP ON BEHALF OF THE STATE OF CALIFORNIA. CHILA SILVA-MARTIN DID A GREAT JOB IN TALKING ABOUT THE FINANCES OF CIRM. DR. MILLAN GAVE A PRESENTATION ON THE PROGRAMS AND THE PROGRESS MUCH LIKE A LOT OF THE MATERIAL YOU HEARD ABOUT TODAY. THAT PRESENTATION WAS VERY ENTHUSIASTICALLY RECEIVED. I SPOKE ABOUT TRANSITION MATTERS; AND AT THE END OF ALL THIS, WE HAD A VERY, VERY POSITIVE RESPONSE FROM THE CONTROLLER AND ALL MEMBERS OF HER GROUP AND WERE UNFAILINGLY IMPRESSED WITH EVERYTHING THAT WE HAVE GOING AND ARE UNANIMOUSLY OF A VIEW THAT CIRM IS DOING GREAT WORK. AND SO I JUST WANTED TO PASS THAT ALONG TO YOU SO

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YOU ARE AWARE OF THAT.

AN ITEM THAT'S GOTTEN ACTUALLY SOME INTERESTING PRESS, AS YOU RECALL, BACK IN SEPTEMBER WE APPROVED, AS I MENTIONED EARLIER, ANOTHER COUPLE OF STEM CELLS ALPHA CLINICS. AND THESE ARE THEMSELVES BEST IN CLASS IN THE WORLD AND HAVE THE IMPRIMATUR OF CIRM ON THEM, SOMETHING THAT GIVES A GREAT DEAL OF COMFORT TO ANYBODY PARTICIPATING IN THE CLINICAL TRIALS THAT ARE BEING UNDERTAKEN AT THOSE FACILITIES.

WE HAVE ON THE FLIP SIDE OF THAT A PROLIFERATION OF UNLICENSED STEM CELL CLINICS, SO-CALLED STEM CELL TOURISM, WHETHER IT'S HERE OR IT'S OTHER STATES OR OTHER COUNTRIES OR WHATEVER, THAT IS RAISING INCREASING ALARM BECAUSE IT IS SELLING A PRODUCT THAT IS UNREGULATED AND UNTESTED AND UNPROVEN TO MANY PEOPLE THAT ARE DESPERATE IN LOOKING FOR ANYTHING. AND YOU'RE STARTING TO SEE BODIES ACKNOWLEDGING THAT AS A MAJOR PROBLEM AND ACTING UPON THAT. SO TOWARDS THAT END, ON OCTOBER 2D, SENATOR'S GOOD FRIEND, SENATOR ED HERNANDEZ, SACRAMENTO, HAD A BILL PASSED WHICH SET UP PROTOCOLS FOR UNLICENSED -- WHAT WILL HAPPEN TO UNLICENSED STEM CELL CLINICS IN THE STATE OF CALIFORNIA, WHICH HAS THE CALIFORNIA MEDICAL BOARD AS THE OVERSEER.

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THERE ARE PENALTIES ATTACHED WHICH GET PROGRESSIVELY WORSE. IT IS ACKNOWLEDGING THE PROBLEM.

I BELIEVE, SENATOR, CORRECT ME IF I'M WRONG, I THINK THIS IS THE FIRST OF ITS KIND IN ANY STATE FOR SUCH LAW PASSED IN ANY STATE IN THE COUNTRY TO TRY TO DEAL WITH THIS ISSUE. AND I'M CERTAIN THAT, AS CALIFORNIA TENDS TO BE, WILL BE THE MODEL OF LEGISLATION IN OTHER STATES TO ADDRESS THIS ISSUE IN A SIMILAR FASHION.

AT THE SAME TIME THE FDA IS CRACKING DOWN ON THIS. THERE HAVE BEEN SOME CELEBRATED INSTANCES OF REAL ABUSE BY UNREGULATED STEM CELL CLINICS THAT YOU RECALL THE STORY OF THE THREE WOMEN WHO WENT TO A CLINIC IN FLORIDA GETTING STEM CELL TREATMENTS FOR MACULAR DEGENERATION, ALL THREE OF WHICH ENDED UP BLINDED BY THE TREATMENTS. THIS IS THE SORT OF THING THAT CAN HAPPEN.

THE FDA ON NOVEMBER 16TH CAME OUT WITH A COMPREHENSIVE NEW POLICY APPROACH TO FACILITATING THE DEVELOPMENT OF INNOVATIVE REGENERATIVE MEDICAL PRODUCTS TO IMPROVE HUMAN HEALTH. THAT'S A MOUTHFUL. THE IDEA IS THAT THEY'RE PUTTING IN PLACE NOW PROCEDURES AND PRACTICES FROM A REGULATORY STANDPOINT THAT WILL FURTHER ADDRESS THE ISSUE. I DON'T THINK ANYBODY BELIEVES THAT WHAT HAS BEEN DONE

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TO DATE IS GOING TO COMPREHENSIVELY TACKLE THIS, BUT THESE ARE MAJOR MOVES THAT ARE GETTING US IN THAT DIRECTION. I'M SURE WE'LL BE HAVING FURTHER DISCUSSION ON THIS TOPIC DOWN THE ROAD. JUST WANTED YOU TO BE AWARE OF THAT.

MR. TORRES: MR. CHAIRMAN.

CHAIRMAN THOMAS: YES, MR. SENATOR.

MR. TORRES: THIS LEGISLATION WAS THE RESULT OF A PROGRAM THAT ED PENHOET, MY PREDECESSOR, AND I PUT TOGETHER FOR THE LEGISLATURE TO ALLOW THEM TO BRING IN SCIENCE AND TECHNOLOGY FELLOWS FROM BIOTECH AND FROM OTHER FIELDS TO EDUCATE THE LEGISLATURE AND TO BE PART OF CERTAIN OFFICES IN THE SENATE AND THE ASSEMBLY.

WELL, DR. HERNANDEZ' FELLOW IS THE ONE THAT HELPED DRAFT THE LEGISLATION ALONG WITH THE HELP OF KEVIN MC CORMACK, WHO'S HERE IN THE BACKGROUND, AND REALLY WAS A STELLAR PERFORMANCE BY A YOUNG VIETNAMESE AMERICAN WOMAN, A FELLOW, A PH.D., WHO WORKED VERY CLOSELY WITH DR. HERNANDEZ. SO IT WAS CLEARLY A VERY INTERESTING COLLABORATION. AND THE FACT THAT I'M JUST PROUD OF THE FACT THAT THE PROGRAM THAT AND ED AND I STARTED WITH A GRANT FROM THE GORDON MOORE FOUNDATION ENDED UP PROVIDING, NOT ONLY REAL FELLOWS FOR THE LEGISLATORS, BUT NOW A

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REAL TRUE MANIFESTATION OF A CONCRETE PROPOSAL WHICH IS NOW LAW IN CALIFORNIA.

CHAIRMAN THOMAS: THANK YOU, MR. SENATOR. DR. STEWARD.

DR. STEWARD: JUST TO ACTUALLY BUILD ON WHAT YOU JUST FINISHED SAYING, I THINK IT'S REALLY IMPORTANT TO TAKE THIS INTO THE SAME SORT OF CONTEXT THAT WE WERE JUST CONSIDERING ABOUT THE FUTURE OF CIRM AND GOING FORWARD IN THE PUBLIC ARENA TO BUILD ON RECREATING PROP 71 IN 2020.

I THINK ONE OF THE REALLY IMPORTANT EDUCATIONAL THINGS THAT WE'RE ALL GOING TO HAVE TO UNDERTAKE IS EXACTLY THIS THING OF DIFFERENTIATING BETWEEN THE SCIENCE OF DEVELOPMENT OF STEM CELL TREATMENTS AND THERAPIES VERSUS THIS OTHER VERY DANGEROUS ASPECT OF THINGS THAT ARE OUT THERE. AND IT'S ALL CALLED STEM CELLS, AND IT'S GOING TO BE REALLY HARD TO WORK ON THAT IN A PUBLIC WHO DOESN'T REALLY PAY TOO MUCH ATTENTION TO THE DETAILS OF SCIENCE.

I WONDER IF THE CIRM SCIENCE TEAM AND SOME OF OUR PUBLIC EDUCATION EFFORTS REALLY MIGHT USEFULLY BE DIRECTED IN THAT. AND I DON'T KNOW QUITE HOW TO DO IT. ENOUGH SAID ABOUT THE DIFFICULTY OF IT, BUT JUST TO SORT OF LAY THAT OUT

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THERE AS SOMETHING THAT WE ALL SHOULD BE THINKING ABOUT. THANK YOU.

CHAIRMAN THOMAS: THANK YOU, DR. STEWARD.

MOVING ON, I WANTED TO NOTE THAT, AS YOU ARE PROBABLY AWARE OF, THIS PAST YEAR HAS SEEN THE ADVENT OF ANOTHER VERY MAJOR SOURCE OF FUNDING FOR SCIENTIFIC RESEARCH HEADED BY THE CHAN ZUCKERBERG INITIATIVE, WHO HAVE DEDICATED $3 BILLION TO CHAN ZUCKERBERG SCIENCE, WHICH, AMONGST OTHER THINGS, 600 MILLION OF THAT HAS GONE TO THE SO-CALLED BIOHUB WHICH IS BASED OUT OF UCSF AND INCLUDES STANFORD AND BERKELEY.

DR. OLSON AND I AND DR. NUGUEN WENT OVER AND MET WITH STEVE QUAKE WHO'S FROM STANFORD WHO IS RUNNING THE BIO HUB. WE HAD AN INTERESTING CONVERSATION. THE POINT OF IT WAS SORT OF UP TO US TO TELL THEM WHAT WE'RE DOING AND TO HEAR WHAT THEY'RE DOING AND TO SEE IF THERE ARE ANY POTENTIAL AREAS OF COLLABORATION. THE AREAS OF FOCUS ARE A LITTLE DIFFERENT FROM WHAT WE'RE LOOKING AT. THEY'RE LOOKING AT HUMAN GENE MAPPING, INFECTIOUS DISEASE, SOME OTHER THINGS, BUT THERE ARE SOME POTENTIAL IDEAS FOR COLLABORATION HERE. AND I THINK THAT THAT IS ALWAYS A GOOD THING.

LAST, BUT NOT LEAST, THERE'S BEEN AN EVENT

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THE LAST FEW YEARS CALLED THE WORLD ALLIANCE FORUM, WHICH IS AN EVENT THAT PULLS TOGETHER MANY SCIENTISTS FROM JAPAN, COMES OVER TO THE STATES TO CONVENE A STEM CELL CONFERENCE OUT IN GOLDEN GATE PARK THAT ADDRESSES A VARIETY OF ISSUES THAT ARE RELEVANT TO THE SPACE. THIS YEAR THEY SORT OF INCREASED THE SCOPE OF WHAT THEY WERE LOOKING AT, NOT JUST STEM CELLS, BUT WHAT THEY CALLED HEALTHCARE GAME CHANGERS.

THEY HAD OVER 300 PARTICIPANTS THERE TO ENGAGE IN A NUMBER OF PANELS AND DISCUSSIONS, BOTH IN REGENERATIVE MEDICINE, BUT ALSO IN THE FIELD OF DIGITAL HEALTH AND HEALTHCARE I.T., GENE THERAPY, AND CANCER IMMUNOTHERAPY. NEIL LITTMAN FROM OUR TEAM WENT OVER REPRESENTING CIRM, PARTICIPATED ON A LIVELY PANEL ON FUNDING INNOVATIONS WITH REPRESENTATIVES FROM ROCHE'S VENTURE FUND, PETER THIEL'S BREAKOUT LABS, DEFTA PARTNERS, SILICON VALLEY BANK, AND PROVIDENCE VENTURES, SO IT WAS SORT OF A GROUP THAT CAME AT THIS FUNDING IDEA FROM A NUMBER OF DIFFERENT PERSPECTIVES. THE PANEL WAS VERY WELL RECEIVED. SO, NEIL, THANK YOU.

THEY ALSO ALWAYS HAVE A NICE EVENT AT THE JAPANESE CONSULATE THE NIGHT BEFORE WHERE THE JAPANESE CONSUL GENERAL HOSTS ATTENDEES OF THE

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CONFERENCE, AND IT'S ALWAYS A GOOD CHANCE FOR NETWORKING AND TALKING ABOUT ISSUES OF THE DAY. SO I JUST WANTED TO LET YOU KNOW THAT.

SO THAT CONCLUDES THE CHAIR'S REPORT. WE WILL NOW -- ANYBODY HAVE ANY COMMENTS, THOUGHTS, ANYTHING ANYBODY WANTS TO SAY?

OKAY. SO I THINK WITH THAT, THAT CONCLUDES THE VARIOUS ACTION ITEMS. WE'RE NOW ON TO REPORTS AND DISCUSSION ITEMS. FIRST UP IS GOING TO BE THE CLINICAL PROGRAM UPDATE. KEVIN MC CORMACK IS GOING TO LEAD US IN THAT DISCUSSION.

MR. MC CORMACK: CHAIRMAN THOMAS, MEMBERS OF THE BOARD, MEMBERS OF THE PUBLIC, AND COLLEAGUES, I HAVE NOTHING TO DO WITH THE CLINICAL PROGRAM, I'M HAPPY TO SAY BECAUSE I'D PROBABLY MAKE A MESS OF IT. I'M THE DIRECTOR OF PATIENT ADVOCATE OUTREACH. ONE OF THE GREAT PRIVILEGES AND PLEASURES OF MY JOB IS I GET TO SEE THE REAL WORLD CONSEQUENCES OF WHAT YOU DO HERE AND THE DECISIONS THAT YOU MAKE HERE AND THE PEOPLE WHOSE LIVES ARE TOUCHED BY THAT.

AND TODAY WE'RE FORTUNATE ENOUGH TO BE HEARING FROM SEVERAL OF THOSE PEOPLE AND THE IMPACT THAT IT'S HAD, THE RESEARCH AND THE FUNDING THAT YOU'VE AWARDED OVER THE YEARS, THE IMPACT IT'S HAD ON THEM IN TERMS LIFE-CHANGING, EVEN LIFESAVING

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TREATMENTS.

WE'RE GOING TO BEGIN WITH ADRIENNE SHAPIRO, WHO MARY BASS TALKED ABOUT EARLIER. AND ADRIENNE IS A REMARKABLE WOMAN ON MANY LEVELS, A CHAMPION OF STEM CELL RESEARCH, BUT ALSO A GREAT ADVOCATE FOR SICKLE CELL DISEASE.

MS. SHAPIRO: HELLO. I'VE GOT A LITTLE BIT OF A SCRATCHY THROAT, SO I APOLOGIZE BEFOREHAND.

IT'S FUNNY BECAUSE YOU GUYS ARE THE FIRST PEOPLE I EVER STOOD UP IN FRONT OF AND ASKED FOR SOMETHING. I WAS HERE IN L.A. WHEN DR. KOHN'S RESEARCH PROJECT CAME BEFORE YOU AND ASKED FOR FUNDING, AND I WAS TERRIFIED. AND I NEVER THOUGHT EVER IN THE WORLD I COULD DO THIS, BUT I WAS NEVER EVER GOING TO DO ANYTHING LIKE IT AGAIN. AND I HAVE TO LET YOU KNOW THAT SINCE THEN, I HAVE SPOKEN FROM ONE ON ONE TO PEOPLE WHO THOUGHT THAT STEM CELL RESEARCH WAS SATANISTIC RITUAL TO BEING IN FRONT OF A CROWD OF, I THINK, 5,000 OR 2,000 JAPANESE SCIENTISTS ALL LOOKING AT ME VERY POLITELY.

SO I SAID, WELL, WHAT COULD TAKE A MOM, BECAUSE I'M JUST A MOM, ON A JOURNEY LIKE THIS? HOW COULD THIS HAPPEN? WHAT HAPPENED? WELL, FIRST, I WANTED MY DAUGHTER TO BE FIXED, AND I HAD DONE ALL MY RESEARCH AND I KNEW THAT STEM CELL WAS GOING TO

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BE THE FIX. WHEN YOU GUYS GOT READY, WHEN YOU HAD THE ABILITY TO TAKE STEM CELLS AND TURN THEM INTO SKIN -- SKIN CELLS INTO STEM CELLS, I CALLED YOU UP AND, "I THINK YOU'RE READY FOR ME," WHICH WAS EVEN STRANGER, WAS THAT YOU GUYS SAID, "YES, COME ON." AND I CAME ON THIS JOURNEY.

SO WHAT ELSE HAVE I LEARNED IN THIS JOURNEY? I LEARNED THAT MY CHILD AND I, BEING FOURTH GENERATION OF LIVING WITH A TERRIBLE DISEASE, WERE NOT ALONE. I LEARNED THAT THERE WERE MOTHERS OUT THERE WHO WERE WATCHING OVER THEIR CHILDREN WHO HAD MUCH WORSE CONDITIONS THAN MY CHILD. I MET MY SUPER HERO FRANCES, WHO EVERY DAY WHEN I GET UP AND I SAY WHAT I'M GRATEFUL FOR, I'M GRATEFUL FOR FRANCES BECAUSE SHE SHOWED ME THAT HAVING LIVED THROUGH MY WORLD'S WORSE NIGHTMARE, THE LOSS OF A CHILD, THAT SHE STILL GETS UP EVERY DAY. AND I CAN GET UP EVERY DAY BECAUSE SHE'S GONE THROUGH IT THREE TIMES.

I HAVE MET SOME OF THE MOST FASCINATING PEOPLE IN THE WORLD. I'VE MET RESEARCH DOCS WHOSE BRAINS OUGHT TO BE LIKE THE SIZE OF THIS BUILDING, BUT WHOSE HEARTS WERE DOUBLE THAT SIZE. I'VE MET PEOPLE WHO SIT, AND SOMETIMES THEY'D MAKE JOKES ABOUT YOU PEOPLE -- SORRY, SCIENTISTS -- ABOUT HOW

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YOU CAN'T SPEAK, YOU CAN'T TALK, BUT WHO SAT WITH ME AND BROKE THEIR SCIENCE DOWN TO THE POINT WHERE I COULD GO IN AND EXPLAIN IT TO FIVE-YEAR-OLDS.

I HAVE BEEN ON VENDOR FLOORS AT CONFERENCES WHERE THEY WILL TAKE THE TIME. I WALK UP, I GO UP, "I'M JUST A MOM, I'M JUST HERE, I WANT TO KNOW WHAT YOU DO." AND THEY'VE TAKEN THE TIME TO EXPLAIN TO ME THE WHOLE PROCESS, ALL THE PROCESSES THAT IT TAKES TO TAKE STEM CELLS FROM ONE PLACE TO ANOTHER PLACE TO ANOTHER PLACE, TO GUARANTEE THAT WHOEVER RECEIVES THAT, WHOEVER RECEIVES THAT MATERIAL IS GETTING A QUALITY PRODUCT.

I'VE GONE FROM BEING A SELFISH MOMMY, AND I'M TELLING YOU SELFISH AS IN MY KID, US, ME, MY FAMILY, TO BEING A MOTHER, A MOTHER WHO SEES A NEW WORLD, NOT JUST FOR HER CHILDREN, BUT FOR SO MANY OTHER CHILDREN, MILLIONS, THOUSANDS, IN WAYS THAT YOU CANNOT COMPREHEND BECAUSE UNLESS YOU'VE LIVED WITH THE IDEA THAT EVERY DAY YOU SHARE WITH YOUR CHILD COULD BE THE LAST. THERE WERE MANY, MANY MOTHERS LIKE ME. AND IT'S AN INTERESTING THING WHAT HOPE DOES.

I'LL JUST GIVE YOU ONE EXAMPLE OF HOPE. AS PART OF MY WORK, I'VE BEEN ADVOCATING WITH PEOPLE WITH SICKLE CELL WHO DON'T HAVE PARENTS. I HAD THIS

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ONE PARTICULAR YOUNG WOMAN I'VE BEEN FIGHTING FOR, GETTING TREATMENT, AND MAKING SURE THINGS WERE OKAY, AND MEETING ALL HER CHALLENGES. AND I HAD GONE TO INTERNATIONAL STEM CELL SUMMIT. AND I WALKED IN THE VENDOR STORE -- I CALL IT A STORE REALLY BECAUSE AT THE END OF IT, THEY DON'T WANT TO CARRY ANY OF THAT STUFF HOME, SO THEY GIVE ME TONS OF IT. I HAVE TO TAKE BOXES OF IT HOME. SO I COME HOME. WHEN I GOT HOME, I FOUND OUT THAT LAKEISHA, AND I'M GOING TO USE HER NAME, WAS IN THE HOSPITAL AND NOT DOING WELL. SO I PACK UP THIS THING, AND THERE'S THIS LITTLE ANIMAL THAT WE USE TO BE LIKE A STEM CELL GUY. AND THEN THERE WAS THIS BIG, HUGE T-SHIRT. AND THEN THERE WERE ALL OF THESE -- JUST A BUNCH OF GOODIES. AND I TOOK THEM TO THE HOSPITAL ROOM, AND I SAID, "YOU'LL NEVER GUESS WHERE I'VE BEEN. LET ME TELL YOU WHO I MET, AND LET ME TELL YOU WHAT'S GOING ON, AND LET ME TELL YOU WHAT THAT MEANS FOR YOU, AND WHAT THAT MEANS FOR YOUR DAUGHTER WHO IS A TRAIT CARRIER, AND THIS WHOLE NEW WORLD THAT'S COMING TO US, IT'S REAL. IT'S COMING TO US."

SHE PUT ON THE T-SHIRT, SHE GOT THE TOYS, SHE WAS SO HAPPY, SHE SAVED PART OF THE STUFF FOR HER KID WHO WAS GOING TO COME AND VISIT. WE HAD A WONDERFUL, WONDERFUL CHAT. BUT HER EYES WERE

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GLEAMING, AND I WAS CRYING BECAUSE WE WERE SHARING THE FACT THAT THERE WAS REALLY, REALLY HOPE WHERE WE KNEW THAT HOPE AND HOPE FOR HER DAUGHTER WHO WASN'T GOING TO CARRY ON.

SO TWO DAYS LATER LAKEISHA GOT HERSELF DISMISSED FROM THE HOSPITAL, PACKED UP ALL HER GOODIES, WENT HOME. I WENT OVER TO VISIT, AND HER CHILD WAS PLAYING WITH THE TOYS AND THE LITTLE PUZZLE THING. AND LAKEISHA WAS REALLY UNWELL. AND WE TALKED ABOUT WHAT STEM CELLS MEANT, NOT JUST TO SICKLE CELL, BUT TO EVERYONE FOR WHEN SHE GOT THE CURE, SHE COULD FIX HER KNEE. WHEN SHE GOT THE CURE, THERE WERE ALL THESE OTHER THINGS THAT HAD GONE WRONG, WHICH WERE KIDNEYS AND THIS, THAT, AND THE OTHER. AND ALL THAT WOULD ULTIMATELY BE FIXED. BUT THE BEST PART OF ALL WAS THAT HER DAUGHTER WOULD NOT HAVE TO SUFFER THROUGH THIS.

SO FOUR AND A HALF HOURS AFTER I HAD THIS DISCUSSION WITH LAKEISHA SHE DIED. AND THE THING ABOUT THAT DEATH WAS, ON ONE HAND, I THOUGHT, OH, MY GOD. THIS IS HORRIFIC, THIS IS HORRIBLE, I'M NOT CUT OUT FOR THIS WORK. I WAS SO CRUSHED. BUT THEN I THOUGHT A DEATH WITH HOPE VERSUS A DEATH THAT WAS JUST SOMETHING THAT SAYS, OKAY, I'M GOING AND THERE'S GOING TO BE NOTHING TO FOLLOW ME. A DEATH

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WITH REAL HOPE. AND I NEED YOU TO UNDERSTAND WHAT THAT MEANS TO THOSE OF US WHO HAVE HAD NO HOPE, THAT THE WORK YOU DO, WHAT YOU SUPPORT. I KNOW IT'S MEASURABLE ON A SPREADSHEET, BUT IT IS REAL AND IT IS TANGIBLE AND IT'S LIKE -- IT'S UNBELIEVABLE. SO THANK YOU. THANK YOU SO VERY MUCH.

(APPLAUSE.)

MR. MC CORMACK: I THINK WHEN ADRIENNE SAYS JUST A MOM, I THINK IT'S ONE OF THE BIGGEST UNDERSTATEMENTS YOU'LL EVER HEAR BECAUSE IT'S MOTHERS LIKE HER WHO ARE HELPING DRIVE THIS AND WHO ARE HELPING CHAMPION WHAT WE DO. AND WE'RE GOING TO HEAR FROM ANOTHER ONE NOW, FRANCES SALDANA.

MS. SALDANA: HI. I'M FRANCES SALDANA. MANY OF YOU I'VE SEEN MANY TIMES BEFORE, AND YOU ALL PROBABLY KNOW THAT HUNTINGTON'S DISEASE IS A GENETIC DISEASE. WHEN I MARRIED THE FATHER OF MY CHILDREN, I HAD NO IDEA WHAT I WAS IN FOR. SO WE IMMEDIATELY WENT INTO HAVING CHILDREN. SO JUST TO GET TO THE BOTTOM OF WHERE THIS ENDED, I HAD ALL MY CHILDREN, THREE CHILDREN, WITHIN A SEVEN-YEAR PERIOD. THEY WERE THE MOST WONDERFUL YEARS A MOTHER COULD HAVE, THE MOST WONDERFUL GIFT.

HUNTINGTON'S DISEASE TOOK ALL THEM AWAY FROM ME WITHIN SEVEN YEARS. THESE ARE MY CHILDREN.

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MY CHILDREN WERE FIGHTERS. AND WE SAID GOODBYE TO MICHAEL TWO MONTHS AGO.

I HAVE A PICTURE HERE OF MY VERY FIRST CIRM MEETING, WHICH WAS EXACTLY SEVEN YEARS AGO. WE HAD SO MUCH HOPE. WHEN THEIR FATHER DIED, THAT WAS DEVASTATING. HE DIED IN '89. WE HAD NO IDEA HOW TO TREAT HUNTINGTON'S. WE DIDN'T KNOW HOW TO TEST. WE HAD NOTHING. WE DIDN'T HAVE THE INTERNET. SO IT WAS A NIGHTMARE FOR US, AND THE WORST NIGHTMARE FOR ME WAS KNOWING THAT MY CHILDREN MAY BE HAVING THIS VERY SAME DISEASE.

SO I TRIED TO MAKE LIFE AS WONDERFUL AS I COULD FOR MY CHILDREN, INVOLVED THEM IN ACTIVITIES, TRIED TO LEARN ALL I COULD ABOUT HUNTINGTON'S, WENT TO THE UCI LIBRARY, CHECKED OUT VOLUMES OF BOOKS, NOTHING ON HUNTINGTON'S, MAYBE A PARAGRAPH. SO WE TRIED TO PRETEND IT WASN'T GOING TO HAPPEN. MY CHILDREN WOULD ASK ME, "MOM, ARE WE GOING TO HAVE DAD'S DISEASE?" I WOULD TELL THEM, KNOWING I WAS LYING TO THEM, "OF COURSE YOU'RE NOT. YOU LOOK LIKE ME. YOU'RE NOT GOING TO HAVE HUNTINGTON'S." SO WE JUST WENT ON WITH LIFE.

MARGIE WAS VERY ACTIVE IN MUSICAL THEATER. SHE LOVED THAT. SO SHE MOVED TO L.A. AND WOULD WRITE HER OWN SCRIPT AND DID HER OWN STUDENT VIDEOS,

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AND SHE WAS JUST SUCH A LOVABLE, VIBRANT DAUGHTER. SHE WAS A SOCIALITE. EVERYTHING HAD TO -- SHE WAS A PRINCESS. THAT'S WHAT SHE WAS. EVERYTHING WAS POSSIBLE. IF YOU EVER SAW THE MOVIE "ENCHANTED," THAT WAS HER. SHE CONVINCED BY YOUNGEST DAUGHTER MARIE, WHO IS NOW 17 YEARS OLD AND SYMPTOMATIC, AND I KNEW, BUT I DIDN'T HAVE THE HEART TO TELL HER, "BABY, I THINK YOU HAVE HUNTINGTON'S DISEASE." THERE WAS NOTHING. WHY SHOULD I TELL HER?

SO SHE WAS IN THE MISS TEEN ORANGE COUNTY BEAUTY PAGEANT AND DID WELL, BUT I KNEW, I KNEW. EVEN WHEN SHE WAS -- WHEN SHE ENTERED, SHE WOULDN'T BE ABLE TO ANSWER THE Q AND A QUESTIONS WELL. AND WHEN SHE DANCED, SHE WAS LIKE ONE OR TWO BEATS BEHIND EVERYBODY ELSE. BUT I JUST ENCOURAGED HER TO DO WHAT SHE WANTED TO DO.

MICHAEL WAS THE ENTREPRENEUR, WANTED TO HAVE HIS OWN RESTAURANT IN MANHATTAN ONE DAY. AND WENT AS A TEENAGER BY HIMSELF WITHOUT MY PERMISSION TO PARIS TO STUDY CULINARY ARTS AND TOOK FRENCH CLASSES. AND THAT LITTLE KID, I COULDN'T STOP HIM. AND I NOW KNOW THAT WAS PART OF THE JUVENILE ONSET OF HUNTINGTON'S DISEASE. THEY'RE FEARLESS. THEY THINK EVERYTHING IS POSSIBLE.

SO HE CAME BACK AND WANTED TO RAISE MONEY

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FAST. SO HE WENT TO ALASKA AND GOT JOBS ON THOSE FISHING EXPEDITIONS.

WHEN DAVID, MY HUSBAND, AND I DECIDED TO GET MARRIED, I ASKED HIM TO COME BACK BECAUSE I WANTED HIM TO WALK ME DOWN THE AISLE AND HE DID. WHEN HE WALKED ME DOWN THE AISLE, HE STARTED TRIPPING. AND AT A TIME WHEN I SHOULD HAVE BEEN SO HAPPY, GETTING MARRIED TO DAVID WHO'S GOING TO BE MY LIFELONG PARTNER, MY MIND IS ON HUNTINGTON'S DISEASE. DOES MY SON HAVE IT?

SO THIS IS WHERE I JUST STARTED -- IN 2000 I JUST WENT FULL SPEED AHEAD TO ADVOCATE FOR HUNTINGTON'S DISEASE AND STARTED THE HDSA ORANGE COUNTY CHAPTER, AND BY NOW MY CHILDREN, ALL THREE OF THEM, WERE PRETTY SYMPTOMATIC. SO YOU KNOW THEY STARTED LOSING FRIENDS. WHEN THEY'RE WELL AND VIBRANT, THEY'VE GOT LOTS OF FRIENDS. BUT THEY STARTED GETTING SICK, FRIENDS GO AWAY.

LOOKING FORWARD TO THE HUNTINGTON'S DISEASE WALKS, THE FUND RAISERS, LOOKING FORWARD TO MARGIE'S BAKE SALE THAT SHE HAD EVERY YEAR, LOOKING FORWARD TO THE BOWL-A-THON. THAT WAS LIKE CHRISTMAS ONE TIME. SO IN 2007, WHEN KEN CERVIN (PHONETIC) AND BOB KLEIN INVITED ME TO ATTEND MY VERY FIRST HD OR CIRM BOARD MEETING, I JUST THOUGHT, OH, MY GOD.

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THERE'S HOPE. THERE'S HOPE. MY CHILDREN ARE GOING TO BE FINE. MY CHILDREN ARE GOING TO BE CURED.

SO HERE WE ARE, DR. PACIFICI, HANS KIERSTEAD, MY DAUGHTER MARGIE, AFTER SHE SPOKE, MYSELF, AND SHE'S SMILING. THERE'S A VIDEO OF HER AT THIS TALK WHERE SHE'S -- THERE'S MOVEMENT ALL OVER THE PLACE, AND SHE TALKS ABOUT HER CHILDREN JUST ARE KEEPING THEIR DISTANCE FROM HER BECAUSE THEY DON'T KNOW WHAT'S GOING ON. THAT WAS DECEMBER 2007. SO THE FIGHT GOES ON.

NOW MARIE IS NO LONGER ABLE TO WALK. AND SINCE I'M STILL EMPLOYED, I CAN'T TAKE CARE OF HER AT HOME. SHE'S SICK AND SHE'S BED-BOUND. SO I PULL HER OF THE CARE HOME AND PUT HER IN A WHEELCHAIR TO GO AND LET STUDENTS AND CHILDREN SEE WHAT HUNTINGTON'S IS AND TO TELL THEM ABOUT IT AND THANK THEM FOR FUND-RAISING FOR HER. HERE SHE IS JUST THANKING ALL THE CHILDREN FOR RAISING FUNDS FOR HUNTINGTON'S DISEASE RESEARCH.

SO HERE'S MICHAEL PASSING OUT WATER. HE CAN'T WALK ANYMORE, BUT HE'S SMILING AND HE'S HAPPY THAT WE'RE DOING ALL THESE THINGS.

AND 2012 I DECIDED I REALLY HAVE A LOT OF FAITH AND HOPE IN THE WORK THAT DR. THOMPSON IS DOING. I WANT TO SUPPORT DR. THOMPSON. I WANT TO

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KNOW THAT NOT 5 PERCENT, 10 PERCENT, 15 PERCENT IS GOING TO RESEARCH. I WANT ALL OF IT TO GO TO RESEARCH, BUT I ALSO WANT FUNDING TO GO TO THE CLINIC AT UCI, WHICH WE STARTED IN 2005, AND HAVE MONEY FOR PATIENTS WHO DON'T HAVE INSURANCE, PATIENTS THAT WANT A BLOOD TEST. SO WE STARTED HD CARE AT UCI IN 2012. AND WE'RE ALL VOLUNTEERS. WE DON'T GET SALARIES OR ANYTHING. SO WE GIVE 75 PERCENT TO LESLIE THOMPSON TO DO HER WORK ON INDUCED PLURIPOTENT STEM CELLS OR ANYTHING SHE NEEDS FOR HER LAB OR IF SHE WANTS TO SEND A POST-DOC TO A CONFERENCE OR WHATEVER. WHATEVER SHE WANTS. IT'S NOT DESIGNATED. SHE CAN DO WHAT SHE WANTS, AND THE SAME WITH NEIL HERMANOWICZ.

SO NOW THE CLOCK IS TICKING, AND MY LITTLE BABY MARIE PASSED AWAY IN 2009. SHE WAS STILL SMILING AND STILL SINGING THE SONGS SHE SANG AS A COUNSELOR AT THE YMCA. SHE WAS SUFFERING SO MUCH. WHEN SHE PASSED AWAY SHE WEIGHED 67 POUNDS, RECURRING SEPSIS, SEIZURES, GRAND MAL SEIZURES, THAT WE HAD TO JUST DO SOMETHING DIFFERENT. DOCTOR TOLD ME, "MRS. SALDANA, TALK TO YOUR DAUGHTER TO ASK GOD TO TAKE HER." THAT WAS THE HARDEST THING I EVER HAD TO DO, BUT I DID. AND SHE NODDED NO, NO, NO BECAUSE SHE WAS A FIGHTER.

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THE NEXT MORNING I WENT TO SEE HER, SHE SMILED AT ME. I ASKED HER IF SHE LOVED ME. SHE NODDED YES. I SAID I LOVE YOU TOO AND SHE PASSED AWAY.

MY DAUGHTER MARGIE WENT THE BEST WAY. THEY DIVIDED US BETWEEN THREE FAMILIES TO GET A PRIVATE CAREGIVER FOR HER, AND SHE WENT TO SLEEP IN 2014, JUST WENT TO SLEEP AND THEN I WAS CALLED. I DIDN'T GET TO BE THERE WHEN SHE TOOK HER LAST BREATH. SHE ALWAYS THOUGHT SHE WAS GOING TO BE CURED. AND HER WORST NIGHTMARE WAS TO THINK THAT HER CHILDREN MIGHT INHERIT HER DISEASE.

WE CONTINUE WITH THE FIGHT IN HER MEMORY AND IN THE MEMORY OF ALL THE FAMILIES THAT I'VE MET, ALL THE DADS AND MOMS AND CHILDREN THAT I'VE MET WHOSE LOVED ONES HAVE DIED.

MY SON, OH, MY GOD. HE WAS THE FIGHTER, AND THEY'RE EVEN DOING STUDY ON HIM AT THE HOSPITAL BECAUSE HE WAS SO SICK. BECAUSE YOU'RE YOUNG, THEIR ORGANS ARE STRONG, BUT THE BRAIN IS DESTROYING THEIR BODY. AND RECURRING SEPSIS, FOUR MONTHS IN AND OUT OF THE HOSPITAL. AGAIN, THEY TOLD ME, "MRS. SALDANA, THERE'S NOTHING WE CAN DO FOR HIM. LET'S PUT HIM IN A HOSPICE. LET'S PUT HIM IN ON COMFORT CARE." SO WE DID. I SAID, "WELL, HOW LONG

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WILL IT TAKE?" "TWO DAYS," HE SAID. NO, 42 DAYS. HIS HEART WAS STRONG. UNTIL I FINALLY GOT DOWN ON MY KNEES AND PRAYED AND ASKED, "GOD, PLEASE TAKE MY SON. HE'S SUFFERING THE WAY YOUR SON DIED. PLEASE TAKE HIM RIGHT NOW." AND AS I LOOKED UP, I SAID, "FRANCES, I THINK THIS IS IT." AND I GOT UP AND HE TOOK HIS LAST BREATH.

SO MY GRANDDAUGHTER NOW WHO WAS LITTLE WHEN ALL THIS STARTED, JUST A LITTLE GIRL FOUR YEARS OLD PASSING OUT FLIERS. SHE'S NOW A YOUNG LADY, 19 YEARS OLD, AND NOW SHE'S AT RISK, BUT SHE'S WILLING. SHE'S WILLING TO JOIN ME AND TO KEEP GOING ON WITH THE FIGHT. THAT WOULD BE THE WORST NIGHTMARE FOR ME IF SHE HAS HUNTINGTON'S, IF SHE TESTS POSITIVE, AND WE STILL HAVE NOTHING. AT THAT POINT I DON'T KNOW IF I WANT TO GO ON LIVING. BECAUSE I GOT TO SEE MY MOTHER-IN-LAW ONLY ONE TIME. MY LATE HUSBAND DIED AT THE AGE OF 42, AND NOW ALL MY CHILDREN ARE GONE. I DON'T WANT TO SEE MY GRANDCHILDREN GONE.

I HAVE SO MUCH FAITH IN THE WORK THAT DR. THOMPSON IS DOING, THE WORK THAT JAN NOLTA IS DOING, ALL THE WORK THAT HD RESEARCHERS ARE DOING. THEY'RE TRULY MY ANGELS. I'VE EVEN SAID MANY TIMES TO LESLIE, JUST THESE PEOPLE, THEY'RE SO NICE. I WORKED IN THE BUSINESS WORLD ALL MY LIFE SINCE I WAS

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20, AND THIS IS NOT THE WAY IT IS IN THE BUSINESS WORLD, BUT YOUR LAB TECHNICIANS, THEY'RE JUST LIKE A DIFFERENT BREED. I THINK IT'S BECAUSE THEY REALLY CARE. THEY WANT TO REALLY HEAL PEOPLE. IN FACT, NINE OF THEM ACTUALLY WENT TO MICHAEL'S BEDSIDE BEFORE HE DIED. THEY WENT JUST A FEW DAYS BEFORE HE PASSED AWAY. THAT'S HOW MUCH THEY CARE, AND THEY'VE GONE TO THE FUNERALS, AND THEY REALLY HAVE BECOME PART OF OUR FAMILY. THEY GO TO OUR FUND RAISERS.

SO I JUST WANT TO THANK YOU ALL BECAUSE THIS IS WHERE HOPE IS. IT'S RIGHT HERE, RIGHT HERE. AND WITH ALL THE WORK THAT OUR RESEARCHER ARE DOING IN THE LABS. WITH THAT SAID, I JUST WANT TO SAY MY CHILDREN WERE FIGHTERS. I SEE THAT PICTURE OF THAT LITTLE GIRL. THAT'S HOW I WANT TO SEE MY GRANDDAUGHTER AND OUR FUTURE GENERATIONS AND FAMILIES WITH HUNTINGTON'S CAN LOOK JUST LIKE. THANK YOU.

(APPLAUSE.)

MR. MC CORMACK: IN THE INTEREST OF BALANCE FROM HEARING FROM TWO JUST MOMS, WE'LL HEAR FROM JUST DAD. THIS IS DAVID NOW.

MR. SALDANA: THANK YOU, KEVIN. THANK YOU, EVERYONE. MY SAME IS DAVID SALDANA. I'M FRANCES SALDANA'S HUSBAND. AND ACTUALLY I WAS ASKED

TO COME UP AND SAY A COUPLE OF WORDS ON BEHALF OF MULTIPLE SCLEROSIS, WHICH I DO NOT HAVE, AND I'LL EXPLAIN MY CONNECTION WITH THAT. BEFORE I DO, AND I'LL KEEP IT SHORT, I'M ALSO HERE AS A WITNESS.

FRANCES AND I ARE CELEBRATING OUR 20TH ANNIVERSARY THIS YEAR. THANK YOU. ACTUALLY IT'S ON CHRISTMAS EVE IS OUR OFFICIAL ANNIVERSARY. BUT WE'VE ALREADY STARTED HAVING A CELEBRATION. WE HAD A BEAUTIFUL DINNER THE OTHER DAY WITH FRIENDS AND FAMILY. BUT I CAN TELL YOU THAT OVER THESE 20 YEARS, I'VE WITNESSED, I'VE WITNESSED HUNTINGTON'S DISEASE. I SAW MY STEPDAUGHTER MARIE. I REMEMBER HAVING TO GO DOWNSTAIRS, AND SHE'S CUDDLING WITH HER BOYFRIEND ON THE COUCH AND SAYING, "IT'S TIME TO GO. IT'S LATE." I SAW HER. SHE'S WALKING IN HER HIGH HEELS AND GETTING AROUND JUST FINE, AND I SAW HER GO FROM THAT TO WHERE SHE WAS FALLING DOWN, FALLING DOWN THE STAIRS, FALLING EVERYWHERE, TO A WHEELCHAIR TO A NURSING HOME TO PASSING AWAY.

MY STEPDAUGHTER MARGIE, FRANCES AND I GOT TOGETHER RIGHT WHEN MARGIE WAS HAVING MY STEP-GRANDCHILDREN. I SAW THEM -- I'VE KNOWN THEM SINCE THEY WERE BRAND-NEW BABIES. AND MARGIE WAS DRIVING THEM AROUND, AND SHE WAS VIVACIOUS, AND SHE COULD DO ANYTHING. ANYTHING SHE WANTED TO DO, SHE

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FOUND A WAY TO MAKE IT HAPPEN. USUALLY FRANCES HELPED HER A LOT, BUT I SAW MARGIE GO FROM THAT TO NOT BEING ABLE TO DRIVE ANYMORE TO BEING WHEELCHAIR BOUND AND THEM, OF COURSE, SHE ALSO PASSED.

AND MICHAEL, I REMEMBER MY FIRST CONVERSATION WITH MICHAEL WAS LETTING HIM KNOW THAT WE WERE GETTING MARRIED. AND HE WAS A COMMERCIAL FISHERMAN. AND WE GAVE HIM A CALL. I THINK HE WAS IN SEATTLE AT THE TIME, AND I JUST REMEMBER HOW HAPPY HE WAS. AND THEN HE CAME DOWN FROM SEATTLE SOON AFTER THAT, ATTENDED OUR WEDDING AS FRANCES DESCRIBED, AND I SAW HIM DECLINE, AND EVENTUALLY HE PASSED AWAY.

SO I'VE SEEN THIS, AND I'VE SEEN THE INDIGNITIES. I'VE SEEN HOW PEOPLE LOOK AT THEM. AND I'VE SEEN HOW THEY'RE TREATED AND HOW FOLKS DON'T UNDERSTAND THE DISEASE. I'VE ALSO SEEN RESEARCH. WHEN FRANCES AND I GOT MARRIED 20 YEARS AGO, SHE WAS VERY HOPEFUL. SHE SAYS, "OH, THERE'S GOING TO BE TREATMENT IN FIVE YEARS," BUT THERE WASN'T REALLY ANYTHING GOING ON 20 YEARS AGO. THERE WAS VERY LITTLE, BUT I'VE SEEN THAT GO FROM VERY LITTLE TO JUST BARELY UNDERSTANDING THINGS LIKE HUNTINGTON'S DISEASE, THE GENE THAT JUST RECENTLY HAS BEEN IDENTIFIED PROBABLY JUST A FEW YEARS BEFORE

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THAT. SO SEEING THE RESEARCH GO FROM BASICALLY NOTHING TO WHERE THERE MIGHT BE A TREATMENT. THERE WAS AN ANNOUNCEMENT JUST A COUPLE DAYS AGO, AND THEN THERE'S OTHER LABORATORIES THAT HAVE SOME REALLY PROMISING RESULTS, DR. LESLIE THOMPSON BEING ONE OF THEM.

OH, WE'RE WATCHING, AND WE'RE DOING EVERYTHING WE CAN TO ASSIST HER. AND WE'RE VERY HOPEFUL. AND I GUESS THAT'S THE THEME BETWEEN ADRIENNE SHAPIRO AND FRANCES IS HOPE.

I WAS ASKED TO PUT IN A WORD ON BEHALF OF VISITORS, ANYTHING TO PROMOTE MULTIPLE SCLEROSIS. AND I WANTED TO DO THAT, AND THERE'S A CONNECTION IN OUR FAMILY WITH MULTIPLE SCLEROSIS AS WELL. AND MY BROTHER-IN-LAW, FRANCES' BROTHER-IN-LAW, HE'S MARRIED TO FRANCE'S SISTER. HE'S GOT A VERY DEVASTATING, PROGRESSIVE FORM OF MULTIPLE SCLEROSIS. AND I'VE WITNESSED HIM. SO I'VE BEEN A WITNESS NOT JUST FOR HD, BUT FOR MULTIPLE SCLEROSIS. I'VE SEEN HIM WHEN HE WAS VIBRANT, ABLE TO GET AROUND EASILY, AND DO ALL KINDS OF RIDING A MOTORCYCLE, GOING ON -- HAD A BOAT TO NOW IT'S GOTTEN TO THE POINT WHERE I HELP HIM. IF WE HAVE A PUBLIC EVENT, I HELP HIM GO -- AND HE HAS TO GO TO THE RESTROOM, I SAY, "DON'T LOCK THE RESTROOM DOOR BECAUSE, IF SOMETHING

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HAPPENS, I NEED TO BE ABLE TO GET IN TO HELP YOU." AND, IN FACT, THAT'S STARTING TO HAPPEN. I HAVE TO GO IN AND HELP HIM. HE CAN'T REALLY STAND UP ANYMORE. PROBABLY HIS DAYS OF GOING TO PUBLIC EVENTS ARE PROBABLY ABOUT OVER. AND IT'S REALLY A SHAME.

WITH HUNTINGTON'S DISEASE, EVERYTHING DEGENERATES, NOT ONLY THE BODY, BUT THE MIND DEGENERATES. WITH MULTIPLE SCLEROSIS, YOUR BODY DEGENERATES, BUT YOUR MIND IS THERE. MY BROTHER-IN-LAW HAS TWO PH.D.'S. HE WAS AN ENGINEER. HE WAS A MATHEMATICIAN. AND THEN BECAUSE OF HUNTINGTON'S DISEASE, BECAUSE HIS NIECES AND NEPHEW HAD HUNTINGTON'S DISEASE, HE GOT ANOTHER PH.D. IN COMPUTATIONAL BIOLOGY, AND HE WORKS FOR DR. LESLIE THOMPSON. HE WORKS IN HER LAB OR HE USED TO WORK IN HER LAB. NOW HE WORKS PART TIME FROM HOME BECAUSE HE CAN'T GET THERE. HE'S BRILLIANT. BUT IT'S ALMOST TO THE POINT WHERE HE CAN'T USE A COMPUTER ANYMORE.

BUT ANYBODY WITH MULTIPLE SCLEROSIS, WITH A DEGENERATIVE DISEASE, THE INDIGNITIES OF LOSING THE USE OF YOUR LIMBS, LOOSING YOUR HANDS, BEING CONFINED TO A WHEELCHAIR, TO A SCOOTER, AND THEN NOT HAVING ANY HOPE THAT THERE'S ANYTHING ON THE

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HORIZON. WITH HIS FORM OF MULTIPLE SCLEROSIS, THERE'S REALLY NOTHING AVAILABLE FOR HIM. AND HIS WIFE, MY SISTER-IN-LAW, ASKED ME, "CAN YOU JUST PUT IN A PLUG FOR MULTIPLE SCLEROSIS?"

I KNOW THAT CIRM HAS FUNDED SOME PROJECTS FOR MS, AND I WANT TO ASK YOU TO CONTINUE TO LOOK AT MS AS A POSSIBLE RECIPIENT OF RESEARCH. THESE DISEASES WHERE YOUR BODY ATTACKS YOUR OWN BODY AND YOU LOSE THE CAPACITY TO FUNCTION, THESE ARE DEVASTATING. IT'S NOT MS. THERE'S SO MANY OTHERS. I THINK YOU CALL THEM AUTOIMMUNE. IS THAT WHAT YOU CALL THESE KIND OF DISEASES? IT'S A HORRIBLE THING, AND I THINK WE ALL KNOW SOMEONE WHO HAS MS, WHO'S HAD MAYBE KIDNEY. THERE'S SO MANY DIFFERENT TYPES OF DISEASES WHERE YOUR BODY JUST TURNS ON ITSELF. SO ANYTHING THAT CIRM CAN DO TO PROMOTE THAT KIND OF RESEARCH, WE'LL BE CHEERING FOR THE RESEARCHERS, FOR YOU. AND FOR MY BROTHER-IN-LAW, WE'RE CONSTANTLY READING AND KEEP OUR EYES OPEN FOR NEW REPORTS OF PROGRESS. AND IF POSSIBLE, IF THERE'S ANYTHING THAT CAN HELP HIM BECAUSE HE'S JUST BEEN ON A DOWNWARD TRAJECTORY, BUT WE WANT TO SEE THAT TURN AROUND. THANKS FOR LISTENING.

(APPLAUSE.)

DR. CARAS: SO I THINK THOSE INCREDIBLY

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MOVING AND HEART-WRENCHING STORIES ARE REAL REMINDERS OF WHY WE'RE ALL HERE, AND I THINK WE'RE ALL STRUGGLING TO HOLD BACK OUR TEARS. THANK YOU.

MEMBERS OF THE BOARD, MEMBERS OF THE PUBLIC, I'M GOING TO BE PRESENTING OUR QUARTERLY CLINICAL UPDATE. AND I'LL BE FOCUSING ON ONCOLOGY.

THIS SLIDE SHOWS CIRM'S ENTIRE --

MY NAME IS INGRID CARAS, AND I'M A MEMBER OF THERAPEUTICS TEAM HERE AT CIRM.

THIS SLIDE WE'RE SHOWING NOW SHOWS CIRM'S ENTIRE CLINICAL STAGE PORTFOLIO. IT CONTAINS 43 CLINICAL TRIALS THAT CIRM HAS FUNDED, 38 OF WHICH ARE CURRENTLY ACTIVE, AND THERE ARE EIGHT PROGRAMS THAT ARE WORKING TOWARDS AN IND FILING. AND, AS YOU CAN SEE, IT'S A HIGHLY DIVERSE PORTFOLIO, AND WE'RE CONTINUING TO BUILD ON THAT.

SO THERE ARE TEN ACTIVE ONCOLOGY CLINICAL TRIALS. AND ON THE NEXT FEW SLIDES, I'M GOING TO GIVE YOU AN UPDATE ON WHAT THEY ARE, AND WHAT THEY COVER, AND WHY WE THINK IT'S AN EXCITING PORTFOLIO.

SO IF WE LOOK AT THESE TRIALS BY THERAPEUTIC MODALITY, WHAT YOU CAN SEE IS THAT THE MAJORITY, SIX OUT OF TEN, ARE CELL THERAPIES. THREE ARE USING A BIOLOGIC AND ONE TRIAL A SMALL MOLECULE. AND TO EXPLAIN WHY CIRM IS FUNDING SMALL MOLECULES

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AND BIOLOGICS, I JUST WANT TO BRIEFLY REMIND YOU ABOUT THE CANCER STEM CELL CONCEPT.

SO IT'S NOW WELL ESTABLISHED THAT HUMAN TUMORS ARE VERY HETEROGENEOUS. NOT ALL CELLS IN THE TUMOR ARE ALIKE. WHAT THE CANCER STEM CELL CONCEPT SAYS IS THAT TUMOR GROWTH IS FUELED BY SMALL NUMBERS OF SELF-RENEWING CANCER STEM CELLS WITHIN THE TUMOR. THESE CANCER STEM CELLS ARE RESISTANT TO CONVENTIONAL THERAPIES LIKE RADIATION AND CHEMOTHERAPY. SO THEY SURVIVE TREATMENT AND CAN THEN REGROW THE TUMOR AND DRIVE RELAPSE AFTER REMISSION AS ILLUSTRATED IN THIS CARTOON.

THIS EXPLAINS WHY TUMORS ALMOST INVARIABLY COME BACK AFTER INITIALLY SUCCESSFUL THERAPY. CANCER STEM CELLS CAN ALSO SPREAD TO DISTANT SITES AND ARE BELIEVED TO DRIVE METASTASES. AND TAKEN TOGETHER, I THINK THIS LEADS TO THE INEVITABLE CONCLUSION THAT CANCER STEM CELLS MUST BE ERADICATED TO ACHIEVE A CURE.

SO BEFORE WE GO TO THE PORTFOLIO, I THINK IT'S RELEVANT TO TAKE A LOOK AT THE EVOLUTION OF CANCER TREATMENT. SO THROUGH MOST OF THE 20TH CENTURY, CANCER WAS TREATED WITH RADIATION AND CHEMOTHERAPY. THESE KILL DIVIDING CELLS, AND SO THEY MOSTLY TARGET CANCER, BUT THEY ALSO KILL SOME

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NORMAL CELLS, SO THEY COME WITH SIGNIFICANT TOXICITY.

IN THE LATE 1990S, THE FIRST TARGETED THERAPIES CAME INTO USE. THESE CAME OUT OF INCREASING UNDERSTANDING OF THE BIOLOGY OF CANCER. THEY'RE MORE CANCER SPECIFIC AND, THEREFORE, LESS TOXIC, AND THEY INCLUDE THERAPEUTIC MODALITIES LIKE THERAPEUTIC ANTIBODIES, SMALL MOLECULES, AND OTHER BIOLOGICS.

SO WE'RE NOW IN THE 21ST CENTURY. AND SO FAR IT'S TURNING OUT TO BE THE AGE OF IMMUNOTHERAPY. SO THERE ARE MANY DIFFERENT WAYS TO APPROACH IMMUNOTHERAPY, BUT THEY ALL AIM TO COOPT AND BOOST THE IMMUNE SYSTEM'S NATURAL CAPACITY TO DETECT AND DESTROY ABNORMAL CELLS. THESE THERAPIES CAN BE HIGHLY SPECIFIC. THEY CAN ALSO BE EXTREMELY POWERFUL. SOME EXAMPLES ARE CHECKPOINT INHIBITORS, WHICH TAKES THE BRAKES OFF THE T-CELL ARM OF THE IMMUNE SYSTEM. ANOTHER EXAMPLE ARE ENGINEERED CAR-T CELLS. AND IN THIS THERAPY THE PATIENT'S OWN T-CELLS ARE ENGINEERED TO EXPRESS A CHIMERIC ANTIGEN RECEPTOR, CAR FOR SHORT, WHICH TARGETS THE T-CELLS TO THE TUMOR AND BOOSTS THE ANTITUMOR RESPONSE.

AS I THINK WE ALREADY HEARD MENTIONED BY DR. MILLAN EARLIER, 2017 WAS A LANDMARK YEAR FOR

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THIS APPROACH WITH THE FIRST FDA APPROVED CAR-T CELL THERAPY CALLED KYMRIAH, AND IT'S INDICATED FOR PEDIATRIC ALL.

SO WITH THAT IN MIND, THIS IS AN OVERVIEW OF OUR CLINICAL TRIALS IN HEMATOLOGICAL MALIGNANCIES, BLOOD CANCERS. THESE ARE ALL EARLY STAGE CLINICAL TRIALS, PHASE 1 OR EARLY PHASE 2S, AND THEY'RE COLOR CODED BY THERAPEUTIC APPROACH. SO THE TWO AT THE TOP -- AND I'LL BE GIVING YOU SOME MORE DETAIL ON ALL OF THESE. ON THE NEXT FEW SLIDES, THE TWO TOP ONES ARE USING AN IMMUNOTHERAPY APPROACH. THE NEXT ONE IS A TARGETED THERAPY AIMED AT CANCER STEM CELLS. AND THE LAST THREE ARE CELL THERAPIES THAT DO NOT DIRECTLY TARGET CANCER, BUT ARE DESIGNED TO PROVIDE IMMUNE SUPPORT FOR PATIENTS WHO ARE HEAVILY IMMUNOSUPPRESSED BECAUSE THEY'RE UNDERGOING AGGRESSIVE CHEMOTHERAPY TO TREAT THEIR CANCER.

AND THESE ARE OUR SOLID TUMOR TRIALS. AND AS YOU CAN SEE, THERE ARE THREE IMMUNOTHERAPY APPROACHES AND ONE TARGETED THERAPY IN CANCER STEM CELLS.

SO WITH THE FIRST CAR-T CELL APPROVAL THIS YEAR, THERE IS A LOT OF EXCITEMENT IN THE FIELD FOR THIS APPROACH. AND CIRM HAS ACTUALLY BEEN

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SUPPORTING THIS TECHNOLOGY FOR SOME TIME. AND SO I NOW WANT TO TELL YOU ABOUT THREE DIFFERENT PROGRAMS IN OUR PORTFOLIO ALL USING ENGINEERED T-CELLS.

SO STARTING WITH THIS TEAM LED BY CHRISTINE BROWN AT CITY OF HOPE WHO IS DEVELOPING A CAR-T THERAPY FOR MALIGNANT GLIOMA, BRAIN CANCER. AS I'M SURE YOU'RE ALL AWARE, BRAIN CANCER IS A HIGHLY LETHAL AND HORRIBLE DISEASE.

THERE ARE A FEW FEATURES ABOUT THIS PROGRAM THAT MAKE IT UNIQUE. FIRST, IT'S TARGETING A SOLID TUMOR; WHEREAS, MOST OF THE SUCCESSES WITH CAR-T CELL THERAPIES TO DATE HAVE BEEN IN BLOOD CANCERS.

SECOND, AS FAR AS I'M AWARE, THIS IS THE FIRST TIME THAT ENGINEERED T-CELLS ARE BEING USED IN THE BRAIN.

AND THIRD, THIS PROGRAM IS FOCUSED ON A POPULATION OF T-CELLS CALLED STEM CELL MEMORY T-CELLS, WHICH CAN SELF-RENEW AND DIFFERENTIATE AND ARE VERY IMPORTANT FOR LONG-TERM PERSISTENCE OF THE CELLS. SO THIS IS SPECIFICALLY DESIGNED TO OVERCOME A PROBLEM THAT'S BEEN SEEN WITH SOME OF THE EARLIER GENERATION CAR-T CELL THERAPIES WHERE THE CELLS DO NOT PERSIST IN THE BODY. AND AS THEY DISAPPEAR, THE TUMORS CAN COME BACK.

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THIS CLINICAL TRIAL IS A PROGRESSION FROM A CIRM EARLY TRANSLATION AWARD. SO CIRM HAS ACTUALLY BEEN SUPPORTING THE APPROACH ALMOST FROM THE BEGINNING. AND THE APPROACH HAS SHOWN SOME VERY EARLY, BUT VERY PROMISING CLINICAL RESULTS WHICH WERE PUBLISHED LAST YEAR IN THE *NEW ENGLAND JOURNAL OF MEDICINE*.

THIS NEXT TRIAL FROM POSEIDA THERAPEUTICS IS SIMILAR TO THE PREVIOUS ONE IN THAT IT'S ALSO A CAR-T CELL THERAPY ALSO FOCUSED ON STEM CELL MEMORY T-CELLS FOR SELF-ASSISTANCE, BUT IT'S TARGETING A DIFFERENT, NOVEL ANTIGEN OR DIFFERENT INDICATION, MULTIPLE MYELOMA. AND THIS WILL BE A FIRST-IN-HUMAN CLINICAL TRIAL. THIS WILL BE THE FIRST TIME THIS PARTICULAR CAR-T IS BEING TESTED IN HUMANS.

AND THIRD IS THIS PROGRAM LED BY ANTHONY RIBAS AT UCLA. THIS TEAM IS ENGINEERING T-CELLS TO TARGET A TUMOR ANTIGEN THAT'S EXPRESSED ON SEVERAL ADVANCED CANCERS, INCLUDING VERY DIFFICULT TO TREAT SYNOVIAL SARCOMA.

THIS TEAM IS ACTUALLY TAKING A DIFFERENT APPROACH TO ADDRESS THE PROBLEM OF CELL PERSISTENCE. SO WHAT THEY'RE DOING IS ENGINEERING BOTH T-CELLS AND HEMATOPOIETIC STEM CELLS AND ARE THEN ADMINISTERING THEM TOGETHER. SO THE RATIONALE HERE

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IS THAT THE T-CELLS WILL PROVIDE AN IMMEDIATE ANTI-TUMOR EFFECT WHILE THE STEM CELLS WILL ENGRAFT AND PROVIDE A RENEWABLE SOURCE OF ENGINEERED T-CELLS FOR A DURABLE, LONG-TERM CURE. THIS IS A VERY NOVEL AND INNOVATIVE APPROACH THAT'S BEING TESTED FOR THE FIRST TIME IN A CLINICAL TRIAL.

OKAY. I WANT TO TURN NOW TO A COMPLETELY DIFFERENT IMMUNOTHERAPY APPROACH, CD47 BLOCKADE. CD 47 IS OVEREXPRESSED ON CANCER AND CANCER STEM CELLS, AND IT'S AN IMPORTANT MECHANISM FOR IMMUNE EVASION FROM MACROPHAGES. CD47 BLOCKADE TAKES THE BRAKES OFF MACROPHAGES AND ENABLES THEM TO ELIMINATE CANCER AND CANCER STEM CELLS AS ILLUSTRATED IN THAT PICTURE ON THE LEFT. THIS IS SIMILAR TO CHECKPOINT INHIBITORS THAT TAKE THE BRAKES OFF T-CELLS.

CD47 BLOCKADE IS A NOVEL IMMUNOTHERAPY APPROACH. AND BECAUSE CD47 IS WIDELY EXPRESSED ON MANY DIFFERENT CANCERS, IT HAS VERY BROAD INDICATIONS SPANNING MULTIPLE TUMOR TYPES.

SO THIS NEXT SLIDE SHOWS THE HISTORY OF CD47 BLOCKADE DEVELOPMENT. AND I WANT TO WALK YOU THROUGH IT BECAUSE I THINK IT REALLY ILLUSTRATES THE IMPORTANCE OF CIRM AND HOW THIS PROJECT HAS BEEN BROUGHT FORWARD.

SO THE STORY BEGAN WITH SOME COMPELLING

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PRECLINICAL DATA FROM THE WEISSMAN LAB AT STANFORD THAT SHOWED THAT CD47 BLOCKADE PREVENTS THE TRANSFER AND PROPAGATION OF HUMAN AML IN MICE, INDICATING THAT IT ELIMINATES THE CANCER STEM CELLS. THEY ALSO SHOWED THAT CD47 BLOCKADE PREVENTS TUMOR GROWTH AND METASTASIS OF SOLID TUMORS IN MICE, ANOTHER INDICATION THAT IT ELIMINATES THE CANCER STEM CELLS.

SO ON THE STRENGTH OF THIS DATA, THE TEAM RECEIVED A DISEASE TEAM I AWARD IN 2010 THAT FUNDED TRANSLATION OF THIS VERY EARLY RESEARCH CONCEPT AND RESULTED IN A SUCCESSFUL IND FILING IN 2014. THE TEAM THEN WENT TO RECEIVE A DISEASE TEAM III AWARD TO FUND A FIRST-IN-HUMAN PHASE 1 TRIAL IN SOLID TUMORS IN THE U.S. AND IN PARALLEL THEY CONDUCTED A SECOND PHASE 1 TRIAL IN AML IN THE UK THAT WAS NOT FUNDED BY CIRM, BUT WAS HEAVILY INFORMED BY THE DATA FROM THE DISEASE TEAM I AWARD AS WELL AS BY EARLY SAFETY DATA COMING OUT OF THE SOLID TUMOR TRIAL.

SO BASED ON THIS PROGRESS, IN 2016 A NEW COMPANY WAS FORMED, FORTY SEVEN INC. THAT HAS LICENSED THE RIGHTS TO THIS TECHNOLOGY FROM STANFORD AND HAS RAISED $150 MILLION OF PRIVATE FUNDS TO HELP DEVELOP IT. CIRM IS CONTINUING TO SUPPORT THE PROGRAM WITH BOTH FUNDS AND WITH OUR EXPERTISE THROUGH OUR CLINICAL ADVISORY PANEL. AND FORTY

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SEVEN CURRENTLY HAS TWO ACTIVE CIRM 2.0 AWARDS TO CONDUCT TWO TRIALS, ONE IN AML THAT'S FOCUSED ON HIGH RISK PATIENTS IN COMBINATION WITH CHEMOTHERAPY, AND THE SECOND TRIAL IN COLORECTAL CANCER IN COMBINATION WITH CETUXIMAB. AND AT THE END OF THIS UPDATE, YOU'LL BE MEETING A PATIENT THAT PARTICIPATED IN ONE OF THESE TRIALS.

THIS SLIDE HIGHLIGHTS TWO DIFFERENT CANCER STEM CELL-TARGETED THERAPIES. ONE FROM THOMAS KIPPS AT UCSD, WHO IS DEVELOPING A MONOCLONAL ANTIBODY APTLY NAMED CIRMTUZUMAB, AND ONE FROM DENNIS SLAMON AT UCLA. BOTH OF THESE PROJECTS TARGET PATHWAYS THAT ARE IMPORTANT FOR THE GROWTH AND SURVIVAL OF CANCER STEM CELLS, AND BOTH OF THEM ARE BASED ON A ROBUST PRECLINICAL PACKAGE SHOWING THAT THEIR THERAPIES PREVENT THE TRANSFER AND PROPAGATION OF HUMAN CANCERS IN MICE BY ELIMINATING THE CANCER STEM CELLS.

BOTH PROJECTS ARE PROGRESSIONS FROM DISEASE TEAM I AWARDS. THE KIPPS TEAM IS CURRENTLY PARTNERED WITH ONCTERNAL THERAPEUTICS, AND THEY ARE CONDUCTING A PHASE 1/2 TRIAL IN CLL, TESTING CIRMTUZUMAB IN COMBINATION WITH IBRUTINIB, AND THE SLAMON TEAM IS COMPLETING A PHASE 1 TRIAL IN ADVANCED SOLID TUMORS.

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AND, LASTLY, THIS SLIDE HIGHLIGHTS TWO CORD BLOOD EXPANSION CELL THERAPIES, ONE FROM NOHLA THERAPEUTICS AND ONE FROM ANGIOCRINE BIOSCIENCE. THESE TEAMS ARE USING TWO COMPLETELY DIFFERENT TECHNOLOGIES TO EXPAND THE NUMBER OF STEM AND PROGENITOR CELLS IN CORD BLOOD. AS I ALREADY MENTIONED, THESE DO NOT DIRECTLY TARGET CANCER, BUT THEY'RE DESIGNED TO IMPROVE OR PROVIDE IMMUNE CONSTITUTION IN PATIENTS AFTER HIGH-DOSE CHEMOTHERAPY.

NOHLA IS CONDUCTING A PHASE 2 TRIAL IN AML PATIENTS, AND ANGIOCRINE A PHASE 1 TRIAL IN HEMATOLOGICAL CANCER.

AND, IN SUMMARY, CIRM HAS A VERY DIVERSE AND, I THINK, EXCITING ONCOLOGY PORTFOLIO. THE MAJORITY ARE CELL THERAPIES. THE PORTFOLIO INCLUDES A NUMBER OF VERY CUTTING-EDGE IMMUNOTHERAPY APPROACHES AS WELL AS SOME CANCER STEM CELL-TARGETED THERAPIES. AND I THINK IT'S REALLY IMPORTANT TO NOTE THAT SEVERAL OF THESE PROGRAMS HAVE BEEN SUPPORTED AND FUNDED BY CIRM PRETTY MUCH FROM INCEPTION.

AND ECHOING WHAT'S BEEN SAID BY OTHER SPEAKERS HERE, I ALSO WANT TO ACKNOWLEDGE THE COURAGEOUS PATIENTS THAT PARTICIPATE IN ALL THESE

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TRIALS, MANY OF THEM KNOWING THAT THEY THEMSELVES MAY NOT BENEFIT, BUT THAT THEIR PARTICIPATION WILL HELP OTHER PATIENTS SOMETIME IN THE FUTURE. SO THANK YOU. THAT'S THE END OF MY PRESENTATION. I'LL BE HAPPY TO TAKE QUESTIONS.

MR. TORRES: WONDERFUL TALK.

CHAIRMAN THOMAS: VERY WELL DONE, INGRID. VERY CLEAR.

DR. CARAS: THANK YOU.

CHAIRMAN THOMAS: VERY INFORMATIVE. THANK YOU AND ALL MEMBERS OF THE TEAM FOR ALL THE HARD WORK ON THIS PORTFOLIO AS WELL AS EVERYTHING ELSE WE DO. SO THANK YOU VERY MUCH.

MR. MC CORMACK: AND WE'RE GOING TO HEAR FROM ONE MORE SPEAKER TODAY. HEARING FROM ADRIENNE AND FRANCES AND DAVID, THEY TALKED ABOUT THE HOPE THAT THE WORK THAT WE DO HERE BRINGS THEM. THE NEXT PERSON WE'RE GOING TO HEAR IS SOMEONE WHO TALKS ABOUT THE LIFE-CHANGING IMPACT FOR THE WORK THAT WE DO HAVE HERE. INGRID TALKED ABOUT THE WORK WITH FORTY SEVEN INC. AND THE WORK THAT DR. IRVING WEISSMAN DID BEFORE THAT HELPED FUND INTO THE CLINIC. AND I'D LIKE TO INTRODUCE YOU NOW TOM HOWING, WHO IS ONE OF THE PATIENTS IN THAT CLINICAL TRIAL.

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WHEN I FIRST TALKED TO TOM ON THE PHONE AND ASKED HIM IF HE WOULD COME HERE, I WASN'T QUITE SURE HOW HE WOULD REACT. AND HE LEAPT AT THE OPPORTUNITY. I THINK IT WAS A CHANCE TO TALK TO YOU AND SAY THANK YOU AND NOT JUST A CHANCE TO GET OUT OF THE WINTER IN MICHIGAN.

MR. HOWING: I'D LIKE TO THANK EVERYONE FOR GIVING ME THE OPPORTUNITY TO BE HERE TODAY, AND HOPEFULLY I WON'T BE TOO EMOTIONAL IN MY OPPORTUNITY TO SPEAK WITH YOU. THANK YOU, DR. THOMAS AND THE REST OF THE BOARD.

AS HE INDICATED, IT IS AMAZING AND SUCH AN HONOR TO BE HERE TODAY. AND IT'S BECAUSE OF YOUR INVESTMENT AND THE TIME IN WORKING WITH FORTY SEVEN THAT I HAVE THE OPPORTUNITY TO BE HERE WITH YOU TODAY. IT IS TRUE THAT I WAS DIAGNOSED WITH COLORECTAL CANCER IN MARCH OF 2015. I WAS TRAVELING FOR BUSINESS AND FOUND MYSELF IN A GREAT DEAL OF PAIN WHEN I WAS IN MANHATTAN, AND I JUST COULDN'T HANDLE IT ANYMORE. SO I FLEW BACK TO GRAND RAPIDS, MICHIGAN, WHERE I'M FROM, AND MY SON TOOK ME RIGHT TO URGENT CARE. AND ONCE I GOT TO URGENT CARE, THEY SAID, YOU'RE GOING RIGHT TO THE HOSPITAL. AND FIVE HOURS LATER ON THE TABLE, AND THEY'RE SAYING, GUESS WHAT. YOU'VE GOT AN ABSCESS, YOU'VE GOT COLORECTAL

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CANCER, AND UNFORTUNATELY IT'S METASTASIZED TO YOUR LIVER AND IT'S ALSO MOVED TO YOUR LUNGS AS WELL.

AS YOU CAN IMAGINE, YOUR WORLD TURNS UPSIDE DOWN. YOU FIND YOURSELF IN A VERY UNUSUAL POSITION. I'M VERY BLESSED IN THAT I HAVE A WONDERFUL PARTNER AND WIFE FOR NOW WE'VE CELEBRATED OUR 25TH ANNIVERSARY. I HAVE THREE FANTASTIC SONS. AND BECAUSE OF MY HEALTH AND BEING IN GOOD HEALTH, AS OF TODAY I WAS ABLE TO ATTEND HIS WEDDING IN JULY. SO IT HAS IMPACTED ME IN SO MANY DIFFERENT WAYS, AND I'VE BEEN TRYING TO IMPACT OTHER PEOPLE THAT ALSO HAVE BEEN DIAGNOSED WITH CANCER AND TRYING TO SHARE WITH THEM THE HOPE AND COURAGE AND THINGS THAT THESE INDIVIDUALS HAVE SHARED. I'M ALWAYS JUST AMAZED AND SO MOVED BY WHAT THEY HAVE BEEN ABLE TO DO.

SO WITH THE FUNDING THAT YOU HAVE PROVIDED FOR FORTY SEVEN, I HAVE BEEN ABLE TO BE ON THAT CLINICAL TRIAL. I'M ACTUALLY, IF WE'RE KEEPING SCORE, I'M NO. 108 OF 122. THEY TRIED TO MOVE ME IN VERY QUICKLY. WHEN I WAS DIAGNOSED IN 2015, I WENT AHEAD AND HAD SOME RESECTIONING DONE. I WENT RIGHT ONTO A CHEMOTHERAPY TREATMENT VERY SIMILAR TO WHAT INGRID WAS SHARING WITH YOU, DEAD ON EXACTLY WHAT I WAS DOING. I WENT THROUGH 12 CYCLES OF THAT, AND I

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HAD SOME MOVEMENT TO WHERE ACTUALLY MY TUMOR WAS REDUCED. SO IT WAS A POSITIVE SIGN. WE WERE THINKING THAT WAS GOING REALLY GREAT, AND BECAUSE OF THE METASTASES, IT CAME BACK.

SO WHAT THEY THEN DID IS I GOT A HEPATECTOMY ON MY LIVER AND THEY REMOVED ONE LOBE OF MY LIVER, THEY DID ABLATIONS ON THE OTHER SIDE OF THE LOBE TO TRY TO REMOVE THE METASTASES, AND THEN I WENT BACK ON CHEMOTHERAPY AGAIN. AND I DID AGAIN A FULL CYCLE, A DIFFERENT COCKTAIL THAT WAS LOADED AS AN ORAL IN ADDITION TO THAT. AND I AGREE. WHEN YOU DEAL WITH CHEMOTHERAPY, YOU TRY TO STAY POSITIVE, YOU SEE HOW DIFFICULT IT IS, HOW IT IMPACTS YOUR LIFE. I WAS CLEAR FOR A LITTLE WHILE AND ALL OF A SUDDEN IT CAME BACK AGAIN.

MY PHYSICIANS SAID WE'VE GOT TO LOOK FOR ANOTHER ALTERNATIVE. LET'S LOOK FOR SOME NEW HOPE. AND THEY HAD AN OPPORTUNITY WITH THE GROUP THAT WORKS WITH FORTY SEVEN, AT LEAST THE CARE SIDE OF IT, A GROUP CALLED START MIDWEST RIGHT IN GRAND RAPIDS. AND THEY SAID, "YES, LET'S GO AHEAD AND GET YOU APPROVED TO BE CONSIDERED FOR THIS CLINICAL TRIAL." I SAID, "YES, LET'S GO AHEAD AND MOVE FORWARD WITH IT." IT'S NEW. I SAID, "YOU KNOW WHAT, LET'S GO AHEAD AND DO THAT." AND I STARTED

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THAT. THEY APPROVED ME IN MAY AND I STARTED THE PROCESS IN JUNE OF THIS YEAR.

AND WHAT THEY FOUND WAS I'VE RESPONDED INCREDIBLY WELL TO IT. RIGHT NOW, IN THE LAST THREE SCANS, WHICH I HAVE EVERY SIX WEEKS, I HAVE THE MRI AND THE CT, AND, OF COURSE, I DO BLOOD WORK WITH MY CDA. AND FOR THE LAST THREE SCANS AND CDA, THEY'RE SHOWING THAT THERE IS NO METASTASES ANYWHERE IN MY BODY. SO I AM VERY FORTUNATE. TODAY I GUESS WE'RE QUITE BLOWN AWAY BECAUSE I GUESS THEY DIDN'T EXPECT IT TO BE SO QUICK OR TO BE THAT COMPLETE.

SO WHERE WE ARE IN THE PROCESS NOW, I DON'T THINK -- OBVIOUSLY IT'S A CLINICAL. NO ONE REALLY KNOWS. WE DON'T KNOW IF IT'S STOPPED IN TIME, AND I'M STILL IN THERAPY. AND IT'S KIND OF NICE TO BE HERE BECAUSE NORMALLY I'D BE GETTING INFUSIONS RIGHT THIS MINUTE EVERY THURSDAY. BUT, AGAIN, WHEN I RECEIVED A CALL FROM KEVIN BECAUSE I OBVIOUSLY WAS VERY FORTUNATE TO HAVE SUCH A POSITIVE RESPONSE, WHEN HE ASKED ME, HE SAID, "WILL YOU BE WILLING TO COME OUT AND TALK TO THE BOARD AND SHARE THIS STORY WITH YOU," I HAD TO KIND OF LAUGH. I'M LIKE GOING, YOU JUST GAVE ME, WITH YOUR CARING COMPASSION AND INVESTMENT, NOT ONLY HOPE FOR MYSELF AND MY FAMILY, BUT FOR EVERYONE ELSE THAT'S IN MY

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POSITION OR WILL BE IN MY POSITION. AND I HAD TO KIND OF LAUGH. YOU'VE GOT TO BE KIDDING ME. I JUST SPENT THREE DAYS AND YOU GAVE ME LIKE 3+ YEARS OF MY LIFE BACK. IT WAS KIND OF IRONIC. IT NEVER DAWNED ON ME. OF COURSE, I'M GOING TO BE HERE. AND I ASKED HIM, I SAID, I WISH I COULD COME TO SEE EVERYONE, WHETHER IT'S THE PEOPLE AT STANFORD, THE PEOPLE AT FORTY SEVEN, THE PEOPLE THAT SPEND DAY AFTER DAY WORKING OVER THE BENCH, LOOKING OVER A MICROSCOPE, CUTTING TISSUE, YOU NAME IT, THE PATHOLOGIST, EVERYONE THAT HAD BEEN SO COMMITTED AND SO DRIVEN BECAUSE THEY WANT TO MAKE A DIFFERENCE IN PEOPLE'S LIVES.

AND WHAT I WANTED TO DO TODAY WAS SHARE WITH YOU AND SAY IT HAS AND I'M PROOF OF THAT. AND WHEN HE ASKED ME, HE NEVER SAID THIS IS WHAT YOU SHOULD SAY OR DO IN THIS GROUP. HOW DO YOU SHARE THAT WITH SOMEONE? THERE'S NO WAY TO SAY THANK YOU. THERE REALLY ISN'T. AND I'VE SPENT WEEKS GOING WHAT AM I GOING TO SAY? HOW CAN I -- THERE'S JUST NO WAY OF ADEQUATELY SAYING HOW MUCH AND HOW MUCH YOU SHOULD CELEBRATE THE POSITIVE IMPACT THAT YOU ARE GOING TO BE MAKING. AND, IF ANYTHING, WHAT IT'S DONE AND THE IMPACT IT'S HAD ON MY LIFE. AND IT'S ALWAYS SO STRANGE FOR ME. IF THERE'S ANYTHING

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THAT'S HUMBLING ABOUT CANCER IS WHEN YOU'RE IN MY POSITION AS A PATIENT AND YOU'VE HAD IT FOR THREE YEARS, YES, THINGS CHANGE AND I DON'T HAVE A COLON, I DON'T HAVE A GALLBLADDER, I DON'T HAVE DA-DA-DA-DA BECAUSE THEY'VE BEEN REMOVED. BUT WHEN YOU'VE ALWAYS BEEN ON THE OTHER -- I THINK THE HARDEST THINGS FOR SOME CANCER PATIENTS, ESPECIALLY PEOPLE I KNOW, IS THAT WHEN YOU'RE ON THE RECEIVING SIDE OF A GIFT LIKE YOU'VE BEEN GIVEN WHEN NORMALLY YOU'RE THE GIVER OR THE ONE THAT'S BEING SELFLESS AND DONATING AND DOING THINGS LIKE THAT YOU DO, NOT FOR FINANCIAL GAIN, BUT BECAUSE YOU KNOW IT'S THE RIGHT THING TO DO AND YOU'RE MAKING A DIFFERENCE IN SUCH A DYNAMIC WAY, IT'S A VERY HUMBLING AND VERY UNIQUE POSITION TO BE IN.

AGAIN, I CAN'T -- IT'S CLICHE. I CAN'T THANK YOU ENOUGH, AND I'M SO HONORED, AGAIN, TO BE WITH ALL OF YOU TODAY AND TO SHARE THIS WITH YOU.

(APPLAUSE.)

MR. HOWING: DOES ANYONE HAVE ANY QUESTIONS OR ANYTHING?

MR. TORRES: NO, BUT I HAVE A STATEMENT FROM A DEAR FELLOW COLON CANCER SURVIVOR AND MY SISTER AS WELL. AND MY SON JUST HAD AT AGE 40 HIS FIRST COLONOSCOPY, CLEAR AS A WHISTLE, AND HE'LL

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HAVE ONE EVERY TWO OR THREE YEARS AS I'VE HAD OVER THE YEARS SINCE I WAS FIRST DIAGNOSED IN '06. I WAS BLESSED. I DIDN'T NEED CHEMO. IT WAS SECTIONED OUT, AND HERE I AM ALMOST, WHAT, 11 YEARS LATER.

SO YOU ARE AN INSPIRATION TO ME BECAUSE YOU WENT THROUGH MUCH MORE THAN I HAD TO GO THROUGH OR MY SISTER HAD TO GO THROUGH. SO THE FACT THAT YOU'VE BEEN BLESSED IN SUCH A WAY IS AN INSPIRATION TO ALL OF US WHO ARE FORMER PATIENTS, BUT FELLOW SURVIVORS. AND NOW I KNOW THAT YOU ARE GOING TO CELEBRATE MANY WEDDING ANNIVERSARIES TO COME BEYOND YOUR 25TH, AND JUST MAKE SURE EVERYBODY THAT'S RELATED TO YOU GETS THEIR COLONOSCOPY AT THE RIGHT TIME.

MR. HOWING: AGAIN, THANK YOU ALL VERY MUCH.

(APPLAUSE.)

CHAIRMAN THOMAS: KEVIN, DOES THAT CONCLUDE? THANK YOU, EVERYBODY, WHO SPOKE FOR SHARING WITH US YOUR STORIES. THEY'RE ALL TREMENDOUSLY COMPELLING, EMOTIONAL, AND DIFFICULT TO STAND UP AND TALK ABOUT. AND YOU ALL DID WONDERFUL JOBS, AND WE SO APPRECIATE YOUR BEING HERE AND SPEAKING TO US. SO THANK YOU ALL.

I THINK NOW WE ARE AT PUBLIC COMMENT.

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THIS IS ON ANY TOPIC THAT ANYBODY CARES TO SPEAK ABOUT. SPEAKERS HAVE THREE MINUTES. IF ANYBODY WANTS TO SPEAK, PLEASE IDENTIFY YOURSELF IN ADVANCE. DO WE HAVE ANY PUBLIC COMMENT HERE? DO WE HAVE ANY PUBLIC COMMENT AT ANY OF OUR SITES ON THE PHONE? OKAY.

WELL, WITH THAT, I WANT TO JUST SAY A COUPLE WORDS. THIS CONCLUDES THE LAST MEETING OF CALENDAR 2017. I THINK IT'S BEEN AN EXTRAORDINARY MEETING, A VERY SUBSTANTIVE MEETING, VERY EMOTIONAL MEETING. I WANT TO PARTICULARLY THANK DR. MILLAN AND THE TEAM AGAIN.

(APPLAUSE.)

CHAIRMAN THOMAS: WHEN SHE WAS GOING THROUGH HER PRESENTATION, OBVIOUSLY IT LOOKED LIKE SOMETHING THAT HAD A LOT OF THOUGHT, BUT I DON'T THINK ANY MEMBERS OF THE BOARD APPRECIATE THE NUMBER OF WOMAN AND MAN HOURS COMBINED FROM MEMBERS OF THE TEAM THAT WENT INTO PUTTING TOGETHER ALL OF THE STRATEGY AND THE PRESENTATION AND EVERYTHING ELSE. SO I DIDN'T WANT TO LET IT PASS WITHOUT COMMENTING ON WHAT GREAT WORK THAT REPRESENTED AND WHAT OBVIOUS WORK, BASED ON WHAT WE'VE HEARD TODAY, CIRM IS DOING.

I THINK OUR STATE OF THE UNION IS GREAT.

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WE HAVE ONLY UPWARDS AND ONWARDS TO GO, GOT GREAT TRAJECTORY, GREAT MOMENTUM. IT'S BEEN A TERRIFIC YEAR. SO THANKS TO EVERYBODY.

AND I'LL JUST CLOSE BY SAYING -- I WOULD LIKE TO CONGRATULATE MY BOSS, DR. BONNEVILLE, AND THANK, AS SENATOR TORRES POINTS OUT, TO THANK ALL THE MEMBERS OF THE TEAM THAT PUT TOGETHER THIS MEETING AND ALL THE MEETINGS WE HAVE. I THINK THIS SITE WORKS VERY WELL. IT'S A WONDERFUL PLACE TO CONVENE, AND A LOT OF HARD WORK GOES INTO PREPARATION AND SETTING UP. SO TO ALL MEMBERS OF THE TEAM RESPONSIBLE FOR THAT.

SO I WILL JUST CONCLUDE BY SAYING I WOULD BE REMISS, MR. JUELSGAARD, MR. ROWLETT, IF YOU'RE STILL ON THE PHONE, IN SAYING THAT I WAS HOPING TO END THIS MEETING WITH A DODGER WORLD CHAMPIONSHIP BANNER ON THE WALL BEHIND ME, BUT DIDN'T QUITE MAKE IT THERE. SPRING TRAINING STARTS IN A COUPLE MONTHS. WAIT TILL NEXT YEAR. SO WITH THAT --

MR. ROWLETT: WELL DONE.

(THE MEETING WAS THEN CONCLUDED AT 02:29 P.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON DECEMBER 14, 2017, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

BETH C. DRAIN, CA CSR 7152
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