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:	OCTOBER 18, 2018 9 A.M.
REPORTER:	BETH C. DRAIN, CSR CA CSR. NO. 7152
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I N D E X

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I N D E X (CONT'D.)

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OAKLAND, CALIFORNIA; OCTOBER 18, 2018

9 A.M.

CHAIRMAN THOMAS: OKAY. GOOD MORNING, EVERYBODY. WE HAVE HAD A BIT OF AN ISSUE UP HERE WITH TRAFFIC. SO IT'S TAKEN A LITTLE BIT LONGER FOR SOME OF THE MEMBERS TO GET TO THE BOARD MEETING HERE, BUT WE'RE ALL SET TO GO. MR. JUELSGAARD IS IN THE ROOM FOR THOSE OF YOU WONDERING WHAT THE APPLAUSE WAS.

SO I'D LIKE TO CALL THE REGULAR MEETING OF ICOC AND APPLICATION REVIEW SUBCOMMITTEE TO ORDER FOR OCTOBER 2018. MARIA, WILL YOU PLEASE LEAD US IN THE PLEDGE OF ALLEGIANCE.

(PLEDGE OF ALLEGIANCE.)

CHAIRMAN THOMAS: MARIA, PLEASE CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: HERE.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: HERE.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: PRESENT.

MS. BONNEVILLE: DEBORAH DEAS. DAVID BRENNER. ANNE-MARIE DULIEGE.

DR. DULIEGE: HERE.
MS. BONNEVILLE: JUDY GASSON.
DR. GASSON: HERE.
MS. BONNEVILLE: DAVID HIGGINS.
DR. HIGGINS: HERE.
MS. BONNEVILLE: STEPHEN JUELSGAARD.
MR. JUELSGAARD: HERE.
MS. BONNEVILLE: SHERRY LANSING.
MS. LANSING: HERE.
MS. BONNEVILLE: LINDA MALKAS.
DR. MALKAS: HERE.
MS. BONNEVILLE: BERT LUBIN.
DR. LUBIN: HERE.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: HERE.
MS. BONNEVILLE: SHLOMO MELMED.
DR. MELMED: HERE.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: HERE.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: HERE.
MS. BONNEVILLE: JOE PANETTA. FRANCISCO PRIETO. ROBERT QUINT. AL ROWLETT. SUZANNE SANDMEYER.
DR. SANDMEYER: HERE.

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MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: HERE.

MS. BONNEVILLE: OSWALD STEWARD. JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: HERE.

MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: HERE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: HERE.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: CAN YOU HEAR ME?

MS. BONNEVILLE: YES. THANK YOU, AL.

WE HAVE A QUORUM.

CHAIRMAN THOMAS: THANK YOU, MARIA. WE'LL GO NEXT TO THE CHAIR'S REPORT. I WANT TO START WITH GETTING YOU ALL AN UPDATE ON THE FUNDRAISING EFFORT. BEFORE I DO THAT, I WANTED TO SORT OF SET THE STAGE FOR IT WITH A FEW COMMENTS THAT WE, AS WE'RE HEADING INTO 2019 SHORTLY, ARE AT AN INFLECTION POINT, AS WE KNOW, AS FAR AS THE FUNDING GOES, BUT WE ALSO SHOULD BE EXTREMELY PROUD OF WHERE THINGS STAND AT THE MOMENT IN TERMS OF WHAT CIRM HAS BEEN ABLE TO ACCOMPLISH TO THIS POINT.

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AND I WAS GOING TO GIVE YOU A FEW STATS, BUT I THOUGHT, IF YOU WOULD INDULGE ME, DR. MILLAN AND I DID AN OP-ED RECENTLY WHICH TALKS ABOUT THE GREAT ACCOMPLISHMENTS OF CIRM. AND I WOULD JUST LIKE, IT'S NOT VERY LONG, BUT I THINK YOU WOULD ENJOY HEARING IT, SO I WANT TO READ IT TO YOU. SO IT GOES AS FOLLOWS:

"BIOTECHNOLOGY WAS BORN IN CALIFORNIA IN THE 1970S BASED ON THE DISCOVERY OUT OF ONE OF ITS UNIVERSITIES. CALIFORNIA IS RESPONSIBLE FOR AN INDUSTRY THAT HAS IMPACTED THE LIVES OF MILLIONS OF PEOPLE WORLDWIDE. IN 2004 THE VOTERS OF CALIFORNIA APPROVED PROPOSITION 71, CREATING THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE AND SETTING THE STATE ON A PATH TO BECOMING A GLOBAL LEADER IN STEM CELL RESEARCH.

"TO DATE THE THERAPIES RESULTING FROM THE INSTITUTE'S WORK ARE NOT JUST CHANGING LIVES; THEY'RE ALREADY SAVING LIVES, LIVES LIKE EVIE VACCARO WHO IS ALIVE TODAY BECAUSE OF A TREATMENT CIRM IS FUNDING. VACARRO WAS BORN WITH SKID, ALSO KNOWN AS BUBBLE BABY DISEASE, AN IMMUNE DISORDER THAT OFTEN KILLS BABIES IN THEIR FIRST TWO YEARS. VACARRO NOW SIX AND DOZENS OF OTHER BABIES WERE GIVEN STEM CELL TREATMENTS THANKS TO THE INSTITUTE.

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ALL ARE SHOWING IMPROVEMENT. SOME ARE NOW SEVERAL YEARS PAST TREATMENT AND CONSIDERED CURED.

"AN ACCIDENT LEFT JAKE JAVIER FROM DANVILLE PARALYZED FROM THE CHEST DOWN ON THE EVE OF HIS HIGH SCHOOL GRADUATION. JAVIER WAS TREATED IN A CIRM-FUNDED CLINICAL TRIAL. TODAY IS HAS REGAINED THE USE OF HIS ARMS AND HANDS, IS DRIVING A CAR, AND IS A SOPHOMORE AT CALPOLY SAN LUIS OBISPO. FIVE OTHER PATIENTS TREATED AT THE SAME TIME AS JAVIER ALL HAVE EXPERIENCED IMPROVEMENTS, MEANING THAT, INSTEAD OF NEEDING ROUND-THE-CLOCK CARE, THEY CAN LEAD INDEPENDENT LIVES.

"A STUDY BY THE TUFTS CENTER FOR THE STUDY OF DRUG DEVELOPMENT ESTIMATED THAT IT TAKES AT LEAST TEN YEARS AND $2.6 BILLION TO DEVELOP ONE SUCCESSFUL DRUG. IN 14 YEARS AND WITH JUST THREE BILLION, CIRM HAS FUNDED A THOUSAND DIFFERENT PROJECTS, ENROLLED 900 PATIENTS, AND SUPPORTED 49 DIFFERENT CLINICAL TRIALS TARGETING DISEASES SUCH AS CANCER, KIDNEY FAILURE, LEUKEMIA. FOUR OF THESE PROGRAMS HAVE RECEIVED AN EXPEDITED DESIGNATION BY THE U.S. FOOD AND DRUG ADMINISTRATION, MEANING THEY COULD GET FASTER APPROVAL TO HELP MORE PATIENTS.

"WE HAVE CREATED A NETWORK OF WORLD-CLASS MEDICAL CLINICS THAT HAVE EXPERTISE IN DELIVERING

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TREATMENTS TO PATIENTS. THE CIRM ALPHA CLINICS OFFER TREATMENTS BASED ON SOLID SCIENCE, UNLIKE THE UNLICENSED CLINICS SPROUTING UP AROUND CALIFORNIA THAT PEDDLE UNPROVEN AND POTENTIALLY HARMFUL THERAPIES THAT COST PATIENTS THOUSANDS OF DOLLARS.

"CIRM HAS SUPPORTED THE CREATION OF 9 A.M. STEM CELL RESEARCH FACILITIES IN CALIFORNIA, ATTRACTED HUNDREDS OF TOP-TIER RESEARCHERS TO CALIFORNIA, TRAINED A NEW GENERATION OF STEM CELL SCIENTISTS, BROUGHT CLINICAL TRIALS TO CALIFORNIA; FOR EXAMPLE, ONE TARGETING ALS OR LOU GEHRIG'S DISEASE, DEPLOYED RIGOROUS SCIENTIFIC STANDARDS AND SUPPORT SO OUR PROGRAMS HAVE A SEAL OF APPROVAL TO ATTRACT 2.7 BILLION IN ADDITIONAL INVESTMENTS FROM INDUSTRY AND OTHER SOURCES.

"WE RECENTLY HAVE PARTNERED WITH THE NATIONAL INSTITUTES OF HEALTH" -- BE HEARING MORE ABOUT THAT LATER -- "TO BREAK DOWN BARRIERS AND SPEED UP THE APPROVAL PROCESS TO BRING CURATIVE TREATMENTS TO PATIENTS WITH SICKLE CELL DISEASE.

"HAVE WE ACHIEVED ALL WE WANTED TO? OF COURSE NOT. THE FIRST DECADE OF CIRM'S LIFE WAS LAYING THE GROUNDWORK, DEVELOPING THE KNOWLEDGE AND EXPERTISE, AND REFINING PROCESSES SO THAT WE CAN TRULY ACCELERATE PROGRESS. AS A LEADER IN THIS

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BURGEONING FIELD OF REGENERATIVE MEDICINE, CIRM NEEDS TO CONTINUE ITS MISSION OF ACCELERATING STEM CELL TREATMENTS TO PATIENTS WITH UNMET MEDICAL NEEDS."

SO THERE, LADIES AND GENTLEMEN, IS A CONCISE SUMMARY OF WHERE WE STAND TODAY. AND I THINK IT'S SOMETHING THAT WE CAN JUSTIFIABLY ALL BE EXTREMELY PROUD OF. SO I JUST WANTED TO CONVEY THAT TO ALL OF YOU.

OKAY. SO WITH RESPECT TO THE BRIDGE FUNDING EFFORT, AS YOU KNOW, OUR GOAL IS BY 2020 TO PROCURE $220 MILLION TO ALLOW CIRM TO CONTINUE ITS PROGRAMS IN A TYPICAL MANNER BETWEEN THE TIME WE ANTICIPATE RUNNING OUT OF MONEY IN LATE 2019 AND THE ELECTION OF NOVEMBER 2020 IN WHICH WE EXPECT THAT THERE WILL BE A CITIZEN-LED INITIATIVE FOR $5 BILLION TO REUP CIRM, AS IT WERE, FOR THE NEXT GENERATION.

SO JUST TO GIVE YOU A FEEL FOR KIND OF THE PROCESS WE'VE BEEN GOING THROUGH, WE HAVE AN IN-HOUSE TEAM THAT MEETS WEEKLY TO DISCUSS PROSPECTS AND STRATEGY. IN THE COURSE OF THAT, WE REFINED THE LOAN PROGRAM THAT WE DISCUSSED AT OUR LAST BOARD MEETING AS A SECOND OPTION TO DONATIONS FOR BRIDGE FUNDING, HAVING CONFIRMED IN THE PROCESS OF THAT

THAT THE IDEA WAS SOUND WITH STATE BOND COUNSEL, THE STATE TREASURER'S OFFICE, AND WITH BOB KLEIN, WHO WILL BE THE AUTHOR OF THE, AS HE WAS WITH THE ORIGINAL PROP 71, WILL BE THE AUTHOR OF THE NEXT PROPOSITION, TO GET HIS AGREEMENT TO PUT IN LANGUAGE THAT WOULD ALLOW FOR BONDS TO BE ISSUED TO REPAY LOANS THAT WE WOULD GET TO ALLOW US TO CONTINUE WITH OUR WORK DURING THIS BRIDGE FUNDING PERIOD.

WE HAVE ANALYZED DOZENS OF POTENTIAL DONORS AND LENDERS BOTH IN TERMS OF THE POTENTIAL ASK THAT WE WILL MAKE AND PARTICULAR SUBJECT MATTERS OF INTEREST TO THOSE SPECIFIC INDIVIDUALS AND HAVE DEVELOPED TAILORED ASKS FOR EACH OF THOSE DONORS OR LENDERS.

WE HAVE, WITH TAILORED ASKS IN HAND, EITHER APPROACHED OR WILL APPROACH THESE POTENTIAL DONORS AND LENDERS AMONG OTHER WAYS AS FOLLOWS: NO. 1 IS EITHER INDIVIDUALLY AND DIRECTLY THROUGH RELATIONSHIPS THAT WE HAVE WITH THE POTENTIAL DONOR, LENDERS IN QUESTION, INDIVIDUALLY THROUGH SUPPORTIVE KEY INTERMEDIARIES WHO ARE CLOSE FRIENDS OR ASSOCIATES OF POTENTIAL FUNDRAISING TARGETS. WE HAVE ALSO ADOPTED A GROUP STRATEGY OF MEETING WITH DINNERS WHERE A NUMBER OF HIGH NET WORTH INDIVIDUALS OR HEADS OF THEIR FAMILY OFFICES COME TOGETHER TO

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HEAR PRESENTATIONS ON CIRM AND ABOUT THE BRIDGE FUNDING EFFORT.

THE PITCHES THAT WE'VE TAILORED, THE SUBJECT MATTER HAS INCLUDED SUCH THINGS AS ADMIN COSTS, UNRESTRICTED FUNDS THAT WOULD COME TO US TO DO WHAT WE WISH TO DO WITH THEM AT OUR DISCRETION, PROJECTS FOR CATEGORIES OF INDICATIONS. SO GO OUT AND SOMEBODY IS INTERESTED IN, SAY, CANCER, DIABETES, WHATEVER, THE PITCH HAS BEEN TAILORED TO THAT PARTICULAR SUBJECT MATTER. AND EVEN MORE SPECIFICALLY, SPECIFIC DISEASES WITHIN CATEGORIES. SO ONE CATEGORY COULD BE GENE THERAPIES, BUT SPECIFIC INDICATION. AND THAT EXAMPLE WOULD BE THE SICKLE CELL INITIATIVE THAT DR. MILLAN AND GABE ARE GOING TO DISCUSS LATER ON.

ANOTHER CATEGORY IS INFRASTRUCTURE. WE ARE CONTEMPLATING POTENTIALLY ADDING SOME NEW ALPHA CLINICS AS WE HAVE HAD DISCUSSIONS ON THAT. WE HAVE ALSO HAD DISCUSSIONS ON A PROGRAM THAT WE SPENT A LOT OF TIME BATTING AROUND POTENTIAL IN-HOUSE THAT WE MIGHT WANT TO DISCUSS AT THE BOARD AT SOME POINT HAVING TO DO WITH THE SYMBIOTIC RELATIONSHIP BETWEEN BASIC RESEARCH AND BIG DATA, WHICH THERE OBVIOUSLY IS A TREMENDOUS OVERLAP AND A NEED TO FUND PROGRAMS THAT ADDRESS THE INTERSECTION OF THOSE TWO.

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WE'VE ALSO TALKED TO A NUMBER OF OUT-OF-STATE, HIGH NET WORTH INDIVIDUALS, AND THE SORT OF PITCH TO THEM IS THEY ARE INTERESTED IN FUNDING REGENERATIVE MEDICINE IN THEIR PARTICULAR AREA OF THE COUNTRY. WE HAVE RECOMMENDED THIS AS SORT OF A SUBJECT MATTER AND GEOGRAPHIC DIVERSITY PLAY FOR THEM. SO THESE DISCUSSIONS ARE ALL IN PROGRESS OR WILL BE IN PROGRESS. AND I JUST WANTED TO LET EVERYBODY KNOW SORT OF WHAT WE ARE THINKING AND HOW WE'VE BEEN SYSTEMATICALLY APPROACHING THIS.

SO IS THERE ANY QUESTIONS ON THAT?

DR. LUBIN: JUST A QUICK QUESTION. IT ALL SOUNDS GREAT. IS THERE SOMEBODY HEADING THE CAMPAIGN? ARE YOU HEADING THE CAMPAIGN?

CHAIRMAN THOMAS: I'M HEADING THE CAMPAIGN. YOU'RE LOOKING AT HIM.

MR. TORRES: BETTER BE CLEAR ABOUT WHAT KIND OF CAMPAIGN YOU'RE TALKING ABOUT.

CHAIRMAN THOMAS: YES, THANK YOU. I GOT THAT FROM ALL SIDES. THIS IS THE BRIDGE FUNDING FUNDRAISING EFFORT. WE'LL STRIKE THE WORD "CAMPAIGN" BECAUSE, AS YOU KNOW, AS A STATE AGENCY, WE CAN'T BE INVOLVED IN CAMPAIGNS. THANK YOU FROM ALL SIDES. OTHER COMMENTS?

DR. MARTIN: FOR A LOAN, IS IT FEASIBLE TO

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NAME A FUNCTION FROM THE LENDER? I KNOW IT'S NOT FOR THE BOND, BUT WHAT ABOUT THE LOAN? COULD THAT BE DIFFERENT?

CHAIRMAN THOMAS: YES. THE QUESTION IS ABOUT NAMING RIGHTS. ABSOLUTELY. THAT'S SOMETHING WE HAVE FACTORED INTO A NUMBER OF THESE DISCUSSIONS. THAT IS, SOME PEOPLE FIND THAT APPEALING; OTHERS DON'T CARE, BUT FOR SOME WE WANT TO MAKE SURE THAT WE MAKE THAT AVAILABLE.

OTHER QUESTIONS? OKAY. THANK YOU.

ON TO THE NEXT PART OF CHAIRMAN'S REPORT. I'VE ASKED -- WE'VE HAD A COUPLE OF VERY INTERESTING MEETINGS INVOLVING EITHER A NUMBER OF MEMBERS OF LEGISLATURE OR INDIVIDUAL MEMBERS, AND I'VE ASKED SENATOR TORRES IF HE'D BE SO KIND AS TO BRIEF YOU ALL ON THOSE. SENATOR TORRES.

MR. TORRES: THANK YOU. YES, WE HAD A VERY PRODUCTIVE HEARING BEFORE THE ASSEMBLY BIOTECH COMMITTEE HEADED BY KEVIN MULLINS WHO REPRESENTS THE SOUTH BAY AREA OF CALIFORNIA, AS WELL AS OTHER MEMBERS OF THE LEGISLATURE, MANY OF WHOM I THINK WERE VERY IMPRESSED WITH THE TESTIMONY OF SOME OF OUR PATIENTS AS WELL AS OUR PATIENT ADVOCATE EXTRAORDINAIRE THERE TO LEND HIS VOICE TO OUR EFFORTS. AND DR. MILLAN ALSO PRESENTED AN EXTENSIVE

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TESTIMONY AS DID DR. JAN NOLTA, WHO FOLLOWED DR. MILLAN.

AND THERE WERE INTERESTING QUESTIONS AND ANSWERS, AND, AGAIN, VERY SUPPORTIVE IN BIPARTISAN WAYS MANY OF THE LEGISLATORS, IN FACT, THE REPUBLICAN MEMBERS, AND I SPOKE AT LENGTH IN TERMS OF WHAT WE NEED TO DO TO FOSTER THIS. ONE OF THE REPUBLICAN MEMBER'S BROTHER IS AN AIDS PHYSICIAN IN NEW YORK. SO THERE WAS REAL INTEREST ON HER PART TO ADVANCE OUR EFFORT. SO ALL IN ALL IT WAS A VERY POSITIVE MEETING.

AND THEN WE HAD SENATOR BEN ALLEN, WHO REPRESENTS THE BEVERLY HILLS AREA, AND A LITTLE PLACE CALLED CEDARS-SINAI IN LOS ANGELES, CAME TO VISIT US AS WELL. AND HE'S VERY INTERESTED IN OUR WORK, AS YOU MIGHT IMAGINE, AND ALSO IS COMMITTED TO WORKING WITH US ON A NUMBER OF ISSUES.

IN RESPECT TO THE STEM CELL CLINICS, AN ISSUE WHICH I'VE BEEN VERY MUCH INVOLVED WITH, PRELIMINARILY ASSEMBLYMEMBER MULLINS HAS AGREED TO OFFER LEGISLATION TO DEAL WITH A CERTIFICATION PROGRAM FOR STEM CELL CLINICS, WHICH I THOUGHT WAS THE MOST APPROPRIATE APPROACH. AND DR. MILLAN AND I HAVE BEEN WORKING ON THAT TO DEVELOP WITH OUR WORKING GROUP, OUR STANDARDS WORKING GROUP, TO

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FIGURE OUT JUST WHAT OUGHT TO BE THE PARAMETERS WHICH OUGHT TO BE THE AGENCY.

AND I'M MEETING AT MY NEXT OTHER HAT, COVER CALIFORNIA, BOARD MEETING WITH OUR SECRETARY OF HEALTH AND WELFARE TO FIGURE OUT JUST WHAT AGENCY HE FEELS MIGHT BE THE APPROPRIATE AGENCY TO CERTIFY THESE STEM CELL CLINICS. AND IT INVOLVES A NUMBER OF ISSUES WHICH WE REALLY CAN'T BE INVOLVED WITH IN TERMS OF LICENSING, BUT WE CERTAINLY CAN BE INVOLVED WITH THE PARAMETERS AND THE DISTINCTIONS THAT WE OUGHT TO RAISE AS TO WHAT CONSTITUTES AN APPROPRIATE STEM CELL CLINIC IN CALIFORNIA. AND WE WILL BE DOING THAT AND I'LL REPORT BACK TO YOU AS SOON AS WE'RE READY WITH THIS LEGISLATION WHICH WE HOPE TO INTRODUCE BY THE END OF JANUARY IN 2019 AND GET IT TO THE NEW GOVERNOR'S DESK IN TIME FOR IT TO BE SIGNED.

CHAIRMAN THOMAS: THANK YOU VERY MUCH, SENATOR TORRES. ANY QUESTIONS OR COMMENTS ON THE SENATOR'S REPORT?

MR. SHEEHY: I HAVE A QUESTION, NOT SO DIRECT TO THE SENATOR, BUT MORE ACTUALLY ABOUT THE OTHER CAMPAIGN. AND WHAT DEGREE OF CERTAINTY DO WE HAVE THAT THAT'S ACTUALLY GOING TO HAPPEN? WHAT IS THE PLANNED INTERFACE BETWEEN THIS BOARD AND THIS

AGENCY AND THE CAMPAIGN? BECAUSE IT SEEMS LIKE THAT'S WHERE ALL OUR EGGS ARE RIGHT NOW. AND IS THERE A PLAN FOR COMMON MESSAGING? YOU KNOW, OBVIOUSLY WE'RE DELIVERING MESSAGES EVERY DAY ABOUT THE WORK THAT WE DO, BUT THE CAMPAIGN WILL HAVE ITS OWN MESSAGING. AND I KIND OF FELT LIKE, WHEN THE *CHRONICLE* ARTICLE RAN, THERE WAS NOT COHERENT MESSAGING BETWEEN THE TWO EFFORTS, AND I THINK THAT THAT PRESENTS PROBLEMS AS WE GET CLOSER TO THE ` CAMPAIGN. AND TO BE PERFECTLY HONEST, THAT CAMPAIGN, FOR ALL RIGHTS AND PURPOSES, STARTS ON NOVEMBER 7TH.

ONCE WE GET OUT OF THIS CYCLE, 2020 LOOMS LARGE IN FRONT OF US. SO IS THERE A PLAN TO PERHAPS DESIGNATE A COMMITTEE OF THE BOARD TO HAVE SOME SORT OF INTERACTION WITH THE CAMPAIGN? THERE SHOULD BE SOME AT LEAST FORMAL DIALOGUE, I THINK. I DON'T THINK THAT'S FORBIDDEN. BUT RIGHT NOW THE CAMPAIGN IS OUT THERE MAYBE AND MAYBE NOT. AND WE HAVE A DUTY AS A BOARD TO BE PREPARED TO TAKE ACTION IF THAT CAMPAIGN DOESN'T MATERIALIZE. AND WE HAVE NO GUARANTEE, NO SENSE THAT IT WILL HAPPEN.

I'M NOT SURE WHAT THE STRATEGY IS FOR GETTING ON THE BALLOT. THERE'S TWO WAYS. ONE IS THROUGH THE LEGISLATURE AND THE OTHER WAY IS THROUGH

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SIGNATURES. WHEN WILL THE CAMPAIGN COMMITTEE BE FORMED? WE'RE TWO YEARS AWAY, AND THESE THINGS, THE LEAD-TIME JUST FOR COLLECTING SIGNATURES, I THINK IT WOULD BE, GIVEN THE LARGE TURNOUT WE'RE GOING TO HAVE IN THE GOVERNOR'S RACE, SIGNATURE COLLECTION EFFORT, IT'S GOING TO BE FAIRLY EXPENSIVE, I WOULD THINK BETWEEN 5 AND 10 MILLION. AND SO HAVING MORE INFORMATION OR SOME SORT OF DIALOGUE ON WHAT THE FUTURE IS AND HAVING SOME SORT OF WAY TO COMMUNICATE BACK AND FORTH SO THAT REALLY WE KNOW AS BOARD MEMBERS WHAT'S HAPPENING. AND I THINK FOR THE PEOPLE, OUR TEAM WHOSE LIVELIHOODS DEPEND ON IT, IT WOULD BE VERY HELPFUL. SO I'M JUST TRYING TO GET A SENSE OF THAT.

CHAIRMAN THOMAS: THANK YOU, MR. SHEEHY.

MR. TORRES: MAY I RESPOND?

CHAIRMAN THOMAS: YES, SENATOR TORRES.

MR. TORRES: NOVEMBER 7TH IS THE START OF ANY CAMPAIGN FOR 2020, WHETHER PRESIDENT OR THIS INITIATIVE. AT THAT POINT WE CAN DETERMINE JUST HOW MANY SIGNATURES WE NEED BECAUSE SIGNATURES REQUIRED FOR AN INITIATIVE ARE BASED UPON THE LAST VOTER TURNOUT IN A GOVERNOR'S RACE. I PRESUME IT'S GOING TO BE HIGHER THAN IT WAS FOR JERRY FOUR YEARS AGO.

SO, NO. 1, FIRST STEP IS ON NOVEMBER 7TH

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GET A SENSE OF WHO VOTED AND THEN FIGURE OUT WHAT THE PERCENTAGE IS. I THINK BOB KLEIN IS WELL AWARE OF HOW WE NEED TO COLLECT SIGNATURES. I HAVE ALWAYS BEEN IN FAVOR OF COLLECTING SIGNATURES BECAUSE THAT'S A GROUNDSWELL OF SUPPORT AND EDUCATION.

MY PROPOSITION 65, WHICH YOU FIND ON THE BACK OF EVERY WINE BOTTLE IN TERMS OF CARCINOGENS, HAD TO BE PASSED BY THE PEOPLE BECAUSE I COULDN'T GET IT THROUGH THE LEGISLATURE. BUT IT TOOK SIGNATURE GATHERING DURING THAT PERIOD OF TIME, LESS EXPENSIVELY OBVIOUSLY IN 1986 THAN IT IS TODAY, BUT CLEARLY SIGNATURE GATHERING IS A PREFERRED METHOD OF EDUCATING THE PUBLIC BECAUSE WHEN YOU GET OUT THERE WITH PEOPLE TALKING TO EACH OTHER CREATES A BUZZ, THAT CREATES AN ONSLAUGHT.

AS TO WHAT OUR RELATIONSHIP SHOULD BE, YES, WE CAN HAVE DISCUSSIONS, AS I'VE BEEN INFORMED, WITH MR. KLEIN WHO CAME TO TESTIFY BEFORE US AND GAVE US SOME VERY ENCOURAGING STATISTICS IN TERMS OF WHERE THE PEOPLE OF CALIFORNIA ARE TODAY. AS YOU KNOW, A POLL IS JUST A SNAPSHOT IN TIME OF THAT PARTICULAR DAY, BUT CLEARLY 70 PERCENT SUPPORT AFTER-THE-PUSH QUESTIONS IS CLEARLY A GOOD OMEN, BUT THAT DOESN'T MEAN IT'S GUARANTEED.

SO THE NATURE OF OUR RELATIONSHIP HAS TO

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BE DELINEATED BY OUR COUNSEL AS TO WHAT WE CAN OR CANNOT DO WITH A CAMPAIGN ONCE IT BEGINS. I'M AN 80-PERCENT EMPLOYEE, AND I'M A PART-TIME EMPLOYEE IN THIS INSTITUTE RECEIVING A SALARY, WHICH HAS NOT BEEN INCREASED SINCE I STARTED IN 2009 THANKS TO THIS BOARD. AND AS A RESULT OF THAT, 20 PERCENT OF MY TIME IS MY OWN, AND I INTEND TO USE IT ALL FOR THIS CAMPAIGN ONCE IT GETS STARTED. SO I WILL BE INTIMATELY INVOLVED WITH THE 20 PERCENT THAT IS NOT ON STATE TIME FOR THIS EFFORT BECAUSE IT IS SO IMPORTANT FOR ALL OF US.

SO THAT'S A PRELIMINARY DISCUSSION. I THINK THERE WILL BE MORE DISCUSSIONS, JEFF, ONCE WE FIGURE OUT JUST WHAT ARE THE LEGAL PARAMETERS THAT WE CAN ABIDE BY IN TERMS OF DISCUSSIONS WITH THE CAMPAIGN AND AS WE MOVE FORWARD.

MR. SHEEHY: IS IT POSSIBLE TO GET A TIMELINE? AND JUST --

MR. TORRES: I JUST SAID WE DON'T HAVE A TIMELINE YET. THE ONLY TIME THAT WE HAVE IS NOVEMBER 7TH AND WE START FROM THERE.

MR. SHEEHY: AND JUST IN TERMS OF SIGNATURES, IT'S ABOUT 20 BUCKS A SIGNATURE NOWADAYS ALL COST IN. SIGNATURES ARE NOT GOING TO BE -- AT LEAST THAT'S WHAT THEY TELL ME COMING OUT OF THE

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LAST CYCLE. SO I JUST -- I GUESS JUST A LITTLE BIT MORE OF A FORMAL STRUCTURE. I MEAN WE ALL OF RIGHTS AND PURPOSES ARE VOLUNTEERS. AND SO WE COULD ADD OUR VOICES TO THOSE WITHIN THE CAMPAIGN AND SOME WAY INFORMALLY TAKE PART, BUT THE CLOCK IS TICKING. AND TWO YEARS IS NOT A LONG TIME IN POLITICS, AND THE AMOUNT OF MONEY IT WOULD TAKE TO RUN A CAMPAIGN IS NOT INSIGNIFICANT.

WHEN WILL THE CAMPAIGN BE OPEN -- CAMPAIGN COMMITTEE BE OPENED IS ONE QUESTION. THE SECOND QUESTION IS HAS THERE BEEN A DECISION MADE TO COLLECT SIGNATURES OR GO TO THE LEGISLATURE AND WHO'S MAKING THAT DECISION? THE NEXT QUESTION IS WHEN IS THE SIGNATURE GATHERING GOING TO START?

THERE'S A LOT OF SIGNIFICANT QUESTIONS. AND, AGAIN, MY CORE POINT IS I THINK WE'VE BUILT AN AMAZING MACHINE HERE AND AN AGENCY. AND I GIVE SO MUCH CREDIT TO THE AMAZING TEAM AT CIRM AND THEIR DEDICATION AND THEIR HARD WORK, BUT THIS IS THEIR LIVELIHOODS. AND I THINK WHEN 2020 STARTS, I THINK EVEN 2019, I WOULD WANT TO KNOW -- IF I HAVE A FAMILY TO SUPPORT, I'D LIKE TO HAVE SOME SENSE OF WHAT IS GOING TO HAPPEN WHEN AND HAVE SOME DEEPER ENGAGEMENT JUST SO I HAD OR DEEPER UNDERSTANDING OF WHAT THE PROCESSES ARE AND WHAT THE PLAN IS SIMPLY

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SO I CAN MAKE MY OWN PLANS.

OUR TEAM HAS REALLY BEEN RECOGNIZED BY NIH FOR THEIR SUPERLATIVE WORK. AND I THINK THIS MAY BE THE FIRST TIME THAT NIH HAS ACTUALLY DELEGATED MANY -- DR. MILLAN WILL TALK ABOUT THIS IN A MINUTE -- MANY OF THEIR FUNCTIONS TO AN OUTSIDE AGENCY, SOME OF THEIR CORE FUNCTIONS, THEY HAVE SO MUCH CONFIDENCE IN THE MACHINE THAT WE'VE BUILT. AND I WOULD HATE TO SEE THAT START TO FADE AWAY BECAUSE OF THE UNCERTAINTY OF WHAT'S GOING ON. THAT'S MY ONLY THING. MAYBE THIS IS -- MAYBE WE CAN HEAR MORE IN DECEMBER.

MR. TORRES: I DON'T THINK ANYONE HERE CAN BE UNMATCHED TO ANYONE HERE IN RESPECT TO OUR CONCERN FOR OUR STAFF AND THEIR LIVELIHOODS. AND THAT'S SOMETHING I TAKE VERY SERIOUSLY, AND CERTAINLY I'M GOING TO DO WHATEVER I CAN TO MAKE SURE THAT INITIATIVE PASSES. I DON'T THINK GOING TO THE LEGISLATURE IS AN APPROPRIATE STEP AND I NEVER THOUGHT THAT. I STILL BELIEVE THAT SIGNATURE GATHERING IS THE BEST WAY TO GO, AND I THINK WE JUST HAVE TO FIGURE OUT WHAT THE BUDGET IS GOING TO BE.

MR. SHEEHY: I'M NOT ADVOCATING FOR GOING TO LEGISLATURE. WE DON'T EVEN HAVE THE FIRM DECISION YET THAT THERE WILL BE A CAMPAIGN.

MR. TORRES: HAS HE TOLD YOU THAT?

MR. SHEEHY: LAST TIME I TALKED TO BOB HE WAS LEANING IN THAT DIRECTION, BUT HE DID NOT SAY THAT HE HUNDRED PERCENT WAS GOING TO RUN A CAMPAIGN. I ASSUME HE IS. EVERYTHING LEADS IN THAT DIRECTION, BUT IT'S UNCERTAINTY.

CHAIRMAN THOMAS: I WOULD ECHO WHAT SENATOR TORRES SAID ABOUT EVERYTHING POINTS TO BOB INTENDING TO DO THAT. THE SIGNATURE GATHERING STRATEGY THAT HE HAS, WHICH SENATOR TORRES COMMENTED ON, YOU MAY RECALL IN PROPOSITION 71, BOB'S TAKE THEN WAS WHATEVER THE NUMBER IS THAT YOU NEED TO GET TO QUALIFY, YOU GO OUT AND GET SEVERAL HUNDRED THOUSAND MORE JUST TO NOT ONLY EMPHASIZE THE POINT, BUT TO GET THAT MANY MORE PEOPLE INVOLVED IN THE DIALOGUE WHO THEN GO OUT AND TALK TO ALL OF THEIR FRIENDS. AND HE VIEWED IT AS STRATEGICALLY A VERY GOOD WAY TO GO TO GENERATE INTEREST IN THE SUBJECT OF THE PROPOSITION.

BUT, JEFF, THAT IS CORRECT, HE HAS NOT SAID DEFINITELY, BUT EVERYTHING POINTS IN THAT DIRECTION. AND WE ARE, UNDER THE GUIDANCE OF MR. TOCHER, ABLE TO PROVIDE HIM WITH INFORMATION AND DATA ON WHAT IS GOING ON AT CIRM WITH RESPECT TO ALL THE PROGRAMS. AND WE ARE DOING THAT AND HAVE BEEN

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DOING THAT.

AND TO YOUR POINT OF TRYING TO GET COMMON MESSAGING, THAT IS DEFINITELY A GOAL THAT WE HAVE GOING FORWARD. SO WE WANT TO BE AS COORDINATED AS WE CAN, AS SUPPORTIVE AS WE CAN, BUT MAKING SURE WE DO SO UNDER THE STRICT GUIDELINE OF MR. TOCHER.

MR. TOCHER: I JUST WANT TO BACK THAT UP. TO YOUR POINT EARLIER, JEFF, ABOUT COORDINATION AND SUCH, I THINK, IF BY COORDINATION YOU MEAN MAKING SURE THAT THE CAMPAIGN AND THE PUBLIC IN GENERAL HAS IMPARTIAL AND ACCURATE INFORMATION ABOUT CIRM'S ACCOMPLISHMENTS OF WHAT CIRM DOES AND HAS DONE, THAT'S ABSOLUTELY TRUE. AND THAT'S ABSOLUTELY SOMETHING THAT THE AGENCY CAN DO NOW AND EVEN THROUGHOUT A CAMPAIGN.

IF BY COORDINATION SOMEONE WERE TO TAKE THAT TO MEAN THAT THERE IS MESSAGING WORKED TOGETHER THAT WOULD HAVE THE EFFECT OF PROMOTING A BALLOT MEASURE OR PROMOTING A CERTAIN OUTCOME ON A BALLOT MEASURE, THAT WOULD BE SOMETHING WE'RE PRECLUDED FROM DOING AND SOMETHING WE WOULDN'T DO.

MR. SHEEHY: HOW WE DESCRIBE THE WORK THAT WE HAVE DONE WITH THE AGENCY, WHAT OUR FUTURE LOOKS LIKE SO THAT WE'RE SPEAKING WITH ONE VOICE ABOUT -- WHICH WE DO ANYWAY.

MR. TOCHER: AND THAT'S SOMETHING THAT HAS PRECEDED THE MEASURE AND PRECEDED THIS EFFORT. WE'VE ALWAYS HAD THAT EFFORT, AND IT HAS ALWAYS BEEN AN ACTIVITY THAT THE AGENCY HAS BEEN ROBUST ABOUT.

CHAIRMAN THOMAS: ANY OTHER COMMENTS ON THIS PARTICULAR TOPIC? OKAY.

JUST A QUICK FEW OTHER POINTS IN THE CHAIR'S REPORT. WE, SINCE THE LAST BOARD MEETING, HAD OUR ANNUAL MEETING OF BRIDGE STUDENTS ON THE ONE HAND AND THE SPARK HIGH SCHOOL STUDENTS ON THE OTHER. AND AS ANY OF YOU WHO HAVE GONE TO THESE MEETINGS WILL ATTEST, THESE PROGRAMS, IN MY OPINION, ARE AMONGST THE BEST THINGS THAT WE FUND HERE. WE EVERY YEAR HAVE HELPED PRODUCE A NEW GENERATION OF POTENTIAL PARTICIPANTS IN THE STEM CELL WORKFORCE THAT ARE REALLY IMPRESSIVE.

IF YOU GO AND LISTEN TO THE PRESENTATIONS THEY MAKE THROUGH THEIR POSTERS AND YOU SIT DOWN AND YOU TALK TO THEM, THEY REALLY ARE NOT ONLY TREMENDOUSLY ENTHUSIASTIC, BUT EXTRAORDINARILY BRIGHT. THAT GOES FOR THE COLLEGE STUDENTS IN THE BRIDGE'S PROGRAM, POSTGRADS, BUT ALSO FOR SURE APPLIES TO THE HIGH SCHOOL STUDENTS WHO EVERY YEAR GO IN TO BEGIN THEIR EIGHT WEEKS HAVING SOME RUDIMENTARY KNOWLEDGE OF STEM CELLS AND COME OUT AT

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THE END OF SUMMER CONFERENCE SOUNDING LIKE BUDDING PH.D.'S. IT'S REALLY SOMETHING THAT'S IMPRESSIVE. AND I WOULD RECOMMEND TO THE BOARD NEXT YEAR WE'LL LET PEOPLE KNOW BECAUSE THESE MEETINGS TEND TO MOVE AROUND. IF YOU HAVE THEM IN YOUR AREA, YOU REALLY SHOULD GO TO ONE OF THESE BECAUSE YOU'LL JUST BE EXTRAORDINARILY IMPRESSED AND REALLY FEEL GOOD ABOUT THE FUTURE OF THE BUSINESS AND FEEL GOOD ABOUT WHAT CIRM IS ENABLING TOWARDS GETTING THAT WORKFORCE IN PLACE.

WE ALSO HAD WHAT WE CALL THE MEETING ON THE MESA DOWN IN LA JOLLA, WHICH IS AN ANNUAL GATHERING OF INDUSTRY. AND IT'S ALWAYS A GOOD BELLWETHER, MUCH AS THE J.P. MORGAN CONFERENCE IS, ABOUT THE STATE OF THE INDUSTRY. THEY'VE HAD IT FOR YEARS NOW AT THE ESTANCIA HOTEL, WHICH A NUMBER OF YOU HAVE STAYED AT PROBABLY, AND THERE WERE SO MANY COMPANIES IN ATTENDANCE AT THIS MEETING, THAT THE BOOTHS WERE NOW OUT IN THE DRIVEWAYS AND THE ROADS LEADING INTO THE HOTEL, AND THEY'VE TOTALLY OUTRUN THE -- OUTGROWN THE PLACE WHERE WE'RE GOING TO HAVE THE CONFERENCE FOR NEXT YEAR. I FORGET, THEY SAID CALABASAS OR SOMETHING, BUT THE ATTENDANCE HERE ON THE INDUSTRY SIDE WAS 25 PERCENT UP FROM LAST YEAR, WHICH IS A HUGE INCREASE. THERE WERE MANY COMPANIES

THAT HAVE CONNECTION TO CIRM-FUNDED RESEARCH. DR. MILLAN WHAT WOULD YOU SAY? HOW MANY WERE THERE AS FAR AS YOU COULD TELL?

DR. MILLAN: AT LEAST TEN TO 9 A.M. REPRESENTED, EITHER ACADEMIC OR INDUSTRY.

CHAIRMAN THOMAS: SO CIRM IS ALWAYS PROMINENTLY FEATURED IN THESE THINGS.

ANOTHER THING, IF YOU HAPPEN TO FIND YOURSELF IN SAN DIEGO IN EARLY OCTOBER, IT'S A GOOD THING TO DROP IN TO SEE. VERY IMPRESSIVE.

LASTLY, JUST WANTED TO REPORT LOS ANGELES COUNTY HAS TAKEN A REAL INTEREST IN PROMOTING BIOSCIENCE AND HAD -- THIS IS THROUGH, IN PARTICULAR, ONE OF THE SUPERVISORS DOWN THERE, MARK RIDLEY THOMAS, AND THEY'VE CONVENED A GROUP CALLED BIOSCIENCE L.A., WHICH IS A MEETING THAT WAS HELD A NUMBER OF WEEKS AGO THAT BROUGHT IN MEMBERS OF INDUSTRY AND ACADEMIA AND FUNDERS ALL WITH SOME SORT OF L.A. COUNTY CONNECTION. EXTREMELY WELL ATTENDED. IT WAS AT LOYOLA MARYMOUNT. AND SUGGESTIVE OF THE RISING LEVEL OF INTEREST AND ENTHUSIASM IN THE FIELD. JOE PANETTA'S GROUP IS PROMINENTLY FEATURED. AND HE, OF COURSE, HAS DONE GREAT WORK IN LEADING INDUSTRY BOTH IN SAN DIEGO AND IN L.A. NOW AND IN THE BAY AREA. AND THIS IS ANOTHER ONE OF THESE

THINGS YOU CAN GO TO TO GAUGE WHERE THINGS ARE GOING. VERY INTERESTING.

WITH THAT, THAT CONCLUDES MY CHAIRMAN'S REPORT. I WOULD NOW LIKE TO TURN IT OVER TO DR. MILLAN FOR THE PRESIDENT'S REPORT.

DR. MILLAN: GOOD MORNING, MEMBERS OF THE BOARD AND THE PUBLIC AND COLLEAGUES. I WILL BEGIN WITH THE PRESIDENT'S REPORT ONCE I GET IT ENABLED TO DO SO.

MR. TOCHER: CHAIRMAN, IF I COULD, JUST INDICATE FOR THE RECORD THAT MEMBERS PRIETO AND STEWARD JOINED DURING THE CHAIRMAN'S REPORT.

CHAIRMAN THOMAS: THANK YOU.

DR. MILLAN: THANK YOU VERY MUCH. WE'RE READY TO START. SO AS WE BEGIN EVERY MEETING, WE BEGIN WITH OUR MISSION, WHICH CONTINUES TO BE TO ACCELERATE STEM CELL TREATMENTS TO PATIENTS WITH UNMET MEDICAL NEEDS, OR ACTUALLY WE CAN EVEN SHORTEN IT FURTHER TO ACCELERATE CURES TO PATIENTS.

BEFORE WE DISCUSS THE PROPOSED BUDGET WE'RE BRINGING TO THE BOARD TODAY, DESCRIBE SOME OF OUR NEW INITIATIVES AND DESCRIBE SOME PROPOSED CONCEPT CHANGES TO SUPPORT OUR UPCOMING ACTIVITIES FOR 2019. I JUST WANTED TO GIVE AN UPDATE ON A VERY BIG PICTURE OVERVIEW ON HOW WE'RE DOING ON THE

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STRATEGIC PLAN WHICH WE LAUNCHED IN 2016.

AS YOU RECALL, WE HAD SIX MAJOR CATEGORIES THAT COMPOSE OUR STRATEGIC PLAN. THE GENERAL PRINCIPLE OF THIS IS TO BUILD A ROBUST PORTFOLIO TO BRING THEM TO THE CLINICS AND TO ACCELERATE DEVELOPMENT. AND SO WHERE WE ARE TODAY IS WE BROUGHT OVER 36 NEW CANDIDATES INTO OUR PIPELINE, WE ARE INCREASING THE PROGRESSION OF THESE PROGRAMS, HAVE SIGNIFICANT NUMBERS OF PROGRAMS GOING FROM ONE STAGE TO THE NEXT, WHICH IS GREATLY ENABLED BY THE NEW INFRASTRUCTURE WHICH HAD BECOME MORE AND MORE HELPFUL FOR OUR PROGRAMS, INCLUDING THE CLINICAL ADVISORY PANEL AND NOW THE NEWLY LAUNCHED TRANSLATIONAL ADVISORY PANEL, AS WELL AS OUR INFRASTRUCTURE PROGRAMS THAT REALLY HELP OUR PROGRAMS OVERCOME BARRIERS AND HELP THEM TO ACCELERATE THE PROGRESSION.

IN ADDITION, IN OUR GOAL OF ENACTING A NEW REGULATORY PARADIGM, WE HAVE ONE OF THE LEADERS IN INTERACTIONS WITH THE FDA IN ENACTING THE NEW REGENERATIVE MEDICINE ADVANCE THERAPIES, AND WE HAVE FOUR OF THOSE PROGRAMS UNDER WAY.

WE NOW HAVE 49 TOTAL CLINICAL TRIALS, 32 NEW CLINICAL TRIALS OF THE TARGET 50. AND I WILL GIVE A LITTLE BIT OF AN UPDATE ON WHERE WE ARE WITH

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THAT LATER.

AND ONE OF THE MOST NOTABLE MEASURES OF WHERE WE ARE, WHAT CIRM'S VALUE PROPOSITION IS, AND WHAT WE HAVE BEEN DOING IN TERMS OF BUILDING AN INDUSTRY, JUST THIS YEAR ALONE APPROXIMATELY $600 MILLION OF ADDITIONAL INVESTMENTS HAVE GONE INTO OUR PORTFOLIO PROGRAMS, BRINGING US TO APPROXIMATELY $2.7 BILLION IN LEVERAGE FUNDING. TWO OF OUR PROGRAMS THAT WE HAD SUPPORTED FROM THE EARLY STAGES AND EARLY RESEARCH AND EVEN IN THE LAB HAVE NOW GONE INTO COMPANIES THAT HAVE RECENTLY SUCCESSFULLY COMPLETED THEIR INITIAL PUBLIC OFFERING AND ARE NOW IN THE PUBLIC MARKET TO HELP SUPPORT THE DEVELOPMENT OF THESE PROGRAMS.

SO THESE ARE ALL JUST INDICATIONS THAT OUR MODEL OF ACCELERATION, DERISKING, AND PARTNERSHIP IS WORKING WELL.

JUST TO GIVE AN UPDATE OF WHAT FUNDING HAS GONE INTO ACCOMPLISHING THESE ACTIVITIES, JUST SHOWING KIND OF AN OVERVIEW OF WHAT THE EXPENDITURES HAVE BEEN THUS FAR INTO OUR FIVE PILLARS OF INVESTMENT SINCE THE INCEPTION OF THE AGENCY. SO OVER $480 MILLION IN INFRASTRUCTURE. THAT INCLUDES NOT ONLY BUILDING INFRASTRUCTURE, BUT PROGRAMS SUCH AS CREATION OF THE IPSC BANK, CREATION OF THE ALPHA

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CLINICS NETWORK, AND ALSO INFRASTRUCTURE TO SUPPORT CLINICAL TRIALS, SUCH AS OUR PARTNERSHIP WITH IQVIA.

ALMOST $900 MILLION INTO DISCOVERY PROGRAMS. AS WE ALL KNOW, GOOD MEDICINE STARTS WITH STRONG SCIENCE. AND SO FROM THE VERY BEGINNING, CIRM'S SIGNATURE HAS ALWAYS BEEN TO SUPPORT TOP TIER, HIGH RISK, BUT HIGH REWARD RESEARCH.

$334 MILLION HAS BEEN INVESTED INTO OUR TRANSLATIONAL PROGRAM. I'D LIKE TO JUST NOTE THAT THE TRANSLATIONAL PIECE OF WHAT CIRM DOES, OF HOW IT PROMOTES TRANSLATION OF DISCOVERIES INTO THE CLINICS, IS SOMETHING THAT IS VERY UNIQUE TO THIS AGENCY. IT IS THE REASON WHY THE NIH WAS ATTRACTED TO US AS A PARTNER IN THE CURE SICKLE CELL INITIATIVE. IT'S SOMETHING THAT'S NOT TAKEN CARE OF BY OTHER ORGANIZATIONS EITHER IN INDUSTRY OR NONPROFIT FUNDING AS ROBUSTLY AS IT IS BY CIRM.

AND WE HAVE HAD AN INCREASING AMOUNT OF INVESTMENT INTO OUR CLINICAL PROGRAMS BECAUSE, AS YOU KNOW, WE HAVE BEEN CONTINUALLY GROWING OUR CLINICAL PORTFOLIO. IN FACT, SINCE THE BEGINNING OF OUR STRATEGIC PLAN, WE'RE PROJECTED BY THE END OF THIS YEAR TO NEARLY TRIPLE OUR CLINICAL PORTFOLIO FROM WHAT IT WAS BUILT UP OVER THE PREVIOUS 9 A.M. YEARS PRIOR TO LAUNCHING, ACTUALLY 16 YEARS PRIOR TO

LAUNCHING THE STRATEGIC PLAN.

$219 MILLION INTO EDUCATION TO BUILD THE WORKFORCE TO START EARLY, AND SENATOR TORRES HAD DESCRIBED SOME OF THOSE PROGRAMS AS WELL AS DR. THOMAS.

YOU WILL RECALL THAT NOVEMBER OF LAST YEAR AND JANUARY EARLY THIS YEAR WE PRESENTED TO THIS BOARD OUR PLANNED TRANSITION PLAN DURING THIS PHASE IN CIRM WHERE WE ARE EXPENDING THE FINAL DOLLARS OF THE PROPOSITION 71 RESEARCH AND ADMINISTRATIVE FUNDS. AND WE ARE CONTINUING TO DO THAT WITH THREE MAJOR BASIC PRINCIPLES. AND THAT IS THAT WE CONTINUE TO EXECUTE ON OUR FIVE-YEAR STRATEGIC PLAN. WE THINK IT'S A GOOD PLAN. WE THINK IT'S ACCOMPLISHING WHAT IT SET OUT TO DO AGAIN AS EVIDENCED BY THE SUCCESSES IN BEING ABLE TO MATURE THE PROGRAMS AND ATTRACT INDUSTRY PARTNERSHIP.

WE ARE CONTINUING TO SEEK TO RETAIN THE TOP TALENT HERE AT CIRM AND MAINTAIN A CRITICAL PERSONNEL LEVEL. AS WE HAD MENTIONED SEVERAL TIMES, CIRM IS A SPECIALTY ORGANIZATION WITH EXPERTISE IN THIS SPACE, EXPERTISE IN TERMS OF THE DEVELOPMENT, EXPERTISE IN TERMS OF THE VALUE PROPOSITION OF A VERY UNIQUE ACCELERATION MODEL SO WE WISH TO RETAIN THAT PERSONNEL LEVEL THROUGHOUT IN THE ENSUING TIME

BETWEEN NOW AND THE 2020 BOND INITIATIVE WHERE WE HOPE THAT WE WILL BE ABLE TO CONTINUE THIS WORK.

WE ALSO ARE CONTINUING NOT ONLY TO MAINTAIN AND PRESERVE CIRM'S VALUE PROPOSITION, BUT DURING THIS TIME PERIOD, WE'RE CONTINUING TO MAKE IMPROVEMENTS IN OUR SYSTEMS. AND WE'RE, ALONG WITH THE BOARD, WHICH YOU WILL SEE IN A LITTLE BIT, ARE CONTINUING TO KEEP AN EYE ON WHERE IS THE FIELD GOING AND WHERE THE OPPORTUNITIES ARE.

SO THIS IS JUST A SNAPSHOT OF OUR CLINICAL PORTFOLIOS. AS YOU CAN SEE, IT IS A VERY DIVERSE PORTFOLIO. FORTY-NINE CLINICAL TRIALS HAVE BEEN FUNDED BY THE AGENCY; APPROXIMATELY 42 OR 43 ARE ACTIVE. THAT IS A HUGE PORTFOLIO BY ANY STRETCH. I WAS AT A RECENT CONFERENCE WITH BIG PHARMA. AND THEIR PORTFOLIOS ARE ABOUT MAYBE EVEN A LITTLE BIT LESS THAN THAT, SO IT'S A VERY ROBUST PORTFOLIO.

SO WITH THAT, I'D JUST LIKE TO GIVE AN UPDATE OF WHAT OUR 2018 RESEARCH BUDGET LOOKS LIKE. ON THE SECOND COLUMN, THE 2018 ALLOCATION, YOU WILL SEE WHAT WAS ALLOCATED INTO THE FOUR PROGRAMS: 130 MILLION INTO CLINICAL, 30 MILLION INTO TRANSLATION, 10 MILLION IN DISCOVERY, AND 750,000 INTO EDUCATION. WE'RE ESTIMATING TO END THE YEAR CLOSE TO BUDGET FOR CLINICAL. WE STILL HAVE A COUPLE OF MONTHS LEFT IN

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THIS YEAR. WE'RE EXPENDING MOST OF THE TRANSLATION BUDGET, 28 MILLION. AND UP FOR DISCUSSION LATER, WE HAD ALLOCATED $10 MILLION FOR DISCOVERY, BUT THE APPLICATION REVIEW SUBCOMMITTEE WILL DISCUSS POTENTIALLY ANOTHER $8 MILLION IN ALLOCATION FOR ADDITIONAL RECOMMENDED DISCOVERY PROGRAMS.

SO BY THE END OF 2018, IN COMPARISON TO WHERE WE STARTED THE YEAR IN JANUARY, WE WILL HAVE APPROXIMATELY $144 MILLION LEFT IN OUR RESEARCH BUCKET. THERE IS AN ESTIMATED APPROXIMATELY $30 MILLION THAT WE EXPECT WILL COME BACK INTO THAT BUCKET. EXCEPT FOR THE EIGHT MILLION THAT IS SUBJECT TO THE MOTION THAT SCOTT TOCHER WILL KIND OF ARTICULATE MUCH BETTER LATER, AN $8 MILLION POTENTIAL SET ASIDE FROM THIS PROJECTED RETURN WILL BE TO REPLENISH THE EIGHT MILLION THAT THE APPLICATION REVIEW SUBCOMMITTEE WILL BE CONSIDERING LATER.

ASIDE FROM THAT, WE'RE JUST PROPOSING A BUDGET BASED ON WHAT IS CASH ON HAND AVAILABLE ON THE RESEARCH BUCKET. SO WITH THE CIRM $144 MILLION RESEARCH BUDGET AND $39 MILLION ADMINISTRATION BUDGET, THE TEAM IS PROPOSING FOR 2019 THAT WE HAVE NO DISCOVERY AWARDS ASIDE FROM THOSE THAT ARE APPROVED FOR FUNDING TODAY. WE WOULD LIKE TO HAVE

BEEN ABLE TO KEEP THE DISC PROGRAM OPEN. WE BELIEVE IT'S CRITICAL. WE BELIEVE IT'S CRUCIAL. WE'RE WORKING HARD TO GET BRIDGE FUNDING FOR THIS BECAUSE IT IS OUR PIPELINE. IT IS WHERE IT ALL STARTS. HOWEVER, GIVEN THE BUDGETARY RESTRICTIONS, WE HAVE ALLOCATED THE REMAINING BUDGET TO TRAN AND CLINICAL PROGRAMS. BUT WITHIN THOSE, AND YOU WILL HEAR A LITTLE BIT MORE LATER WHEN DR. SAMBRANO PRESENTS THE PROGRAM ANNOUNCEMENT CHANGES, WE ARE PROPOSING A CHANGE IN ELIGIBILITY FOR TRAN AND CLIN1 THAT BRINGS IT EVEN TIGHTER INTO THE MIDDLE OF KIND OF THE CIRM MISSION, WHICH IS TO SUPPORT STEM CELL TREATMENTS. AND IN ADDITION TO THAT, AS PROPOSED BY THE SCIENCE SUBCOMMITTEE, WE'RE ALSO PROPOSING TO INCLUDE, IN ADDITION TO EX VIVO GENE THERAPY, IN VIVO GENE THERAPY AS A VITAL RESEARCH OPPORTUNITY BASED ON WHERE THE FIELD IS TODAY.

I WOULD LIKE TO TAKE A MINUTE JUST TO GIVE KIND OF A PERSPECTIVE ON THIS. YOU'VE ALL HEARD ABOUT EVIE WHO HAS SEVERE COMBINED IMMUNO DEFICIENCY, WHICH IS A CELL/GENE THERAPY WHICH HAS LED TO CURES OF EVIE, AND APPROXIMATELY 40 OTHERS HAVE HAD RESPONSES TO THIS TREATMENT. SO IT'S A VERY STRONG PROOF OF CONCEPT FOR THE POWER OF GENE MEDICINE.

YOU'VE ALSO HEARD THAT AT THE END OF LAST YEAR, THE THREE FIRST CELL/GENE PRODUCTS WERE APPROVED BY THE FDA, TWO WITH CAR-TS AND ONE FOR A MONOGENIC EYE DISEASE. SO THE IDEA OF GENE MEDICINE IS HERE.

NOW, ONE CAN SAY, WELL, ISN'T IT MATURE ENOUGH? WON'T INDUSTRY TAKE IT UP? AND THE ANSWER IS ITS STILL EARLY; AND, NO, CIRM STILL HAS AN IMPORTANT ROLE IN TERMS OF DERISKING.

IN ADDITION, MOST OF THESE TARGETS THAT ARE AMENABLE TO GENE MEDICINE ARE RARE DISEASES. SO THEY'RE NOT THE TYPE OF DISEASES THAT PHARMA IS GOING TO GO AFTER. THEY'RE TOO SMALL IN NUMBER, TOO SMALL A MARKET SIZE. BUT WHEN ONE THINKS ABOUT IT, THERE ARE APPROXIMATELY 10,000 MONOGENIC DISEASES THAT CAN BE TARGETED WITH GENE THERAPY. AND THE GENETIC ALLIANCE HAS ACTUALLY ESTIMATED 300 MILLION PEOPLE AROUND THE WORLD THAT ARE AFFECTED BY 7,000 RARE DISEASES. SO IN AGGREGATE THIS IS A HUGE NUMBER, WHICH I THINK CIRM HAS A REALLY IMPORTANT ROLE IN CONTINUING TO PUSH THE SCIENCE FORWARD IN GENE MEDICINE, WHICH IS A NATURAL EXTENSION. IN FACT, THE GENE MEDICINE IS TARGETING CELLS.

SO THAT IS JUST KIND OF A CONTEXT. YOU WILL HEAR A LITTLE BIT MORE ABOUT THAT LATER, AND IT

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WILL BE DISCUSSED, I'M SURE, LATER.

AND THEN I JUST WANTED TO GIVE AN UPDATE. SO WHEN WE FIRST LAUNCHED THE STRATEGIC PLAN, WE WENT BOLD AND WE WANTED 50 NEW CLINICAL TRIALS. AND WE ARE ON PACE FOR THAT. WE FUNDED 32 NEW CLINICAL TRIALS, BRINGING US UP TO A TOTAL OF 49. WHERE WE ARE WITH THE BUDGET, MOST LIKELY WE WILL BE ONLY BE ABLE TO REALLY ACHIEVE ABOUT 43 OR 45 NEW CLINICAL TRIALS, WHICH IS STILL REMARKABLE. BUT IT'S JUST BY WAY OF UPDATE; AND, OF COURSE, IF WE GET NEW FUNDS FLOWING IN AS WELL AS MERITORIOUS PROJECTS, THAT WILL IMPROVE OUR ABILITY TO FUND MORE.

BUT ONE, I THINK, ADVANCE IS THIS PARTNERSHIP WITH THE NHLBI FOR SICKLE CELL CURES INITIATIVE IS THAT IT DOES LEVERAGE OUR FUNDS. SO FOR OUR DOLLARS GOING INTO SUPPORTING CLINICAL PROGRAMS TO THIS VERY IMPORTANT TARGET, NIH WILL MATCH THOSE FUNDS. SO APPROXIMATELY FOR EVERY ONE CLINICAL TRIAL THAT WE COULD HAVE FUNDED, WE MAY BE ABLE TO FUND TWO OR MORE. SO THAT'S VERY, VERY EXCITING FOR US.

AND THEN ONE OF THE THINGS I JUST WANTED TO BRING UP, AND GABE THOMPSON WILL GIVE MORE OF AN OVERVIEW OF HOW WE'RE GOING TO WORK WITH NHLBI ON THIS INITIATIVE, IS I THINK EVERYBODY HAS A LOT OF

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ENTHUSIASM FOR THIS FOR A REASON. SICKLE CELL DISEASE IS SOMETHING WE SHOULD BE ABLE TO CURE GIVEN THE ADVANCEMENTS THAT WE'VE HAD IN GENE MEDICINE AND CELL THERAPY. AND SO WE ARE PLEASED THAT THE NIH RECOGNIZES THE IMPORTANCE OF PARTNERING WITH US, AND WE'RE REALLY EXCITED TO LAUNCH THIS INITIATIVE.

SO FAR WITH THE REMAINING ADMINISTRATIVE BUDGET AND OUR PLANNING, WE DO BELIEVE WE WILL BE ABLE TO PROVIDE SUFFICIENT STAFFING TO MANAGE ALL CIRM AWARDS THAT ARE GIVEN OUT WITH THE REMAINING BUDGET.

SO WITH THAT BACKGROUND, WE'RE PROPOSING THE RESEARCH BUDGET ALLOCATION FOR 2019 AS SHOWN HERE: $93 MILLION FOR CLIN1 AND CLIN2 AWARDS, WITH A SET ASIDE FOR $30 MILLION FOR THE SICKLE CELL JOINT INITIATIVE WITH THE NIH, NHLBI, $20 MILLION FOR TRAN, AND $600,000 FOR THE EDUCATION BUDGET TO FUND ALREADY COMMITTED ACTIVITIES IN OUR EDUCATIONAL PROGRAMS AND OUR ALPHA CLINICS.

IF THERE ARE NO QUESTIONS, I DON'T KNOW, MR. TOCHER, IF WE TAKE A MOTION HERE. OR, MR. SHEEHY, IF YOU'D LIKE TO HAVE A DISCUSSION FIRST ABOUT OTHER MATTERS BEFORE.

MR. SHEEHY: WELL, IT MIGHT BE HELPFUL TO TALK A LITTLE BIT ABOUT THE EIGHT MILLION, WHICH WE

HAD A VERY GOOD TRANSLATION ROUND IN WHICH WE BASICALLY HAD DOUBLED THE NUMBER OF PROJECTS THAT WE HAD FUNDING FOR. AND I THINK THAT THEY SCORED VERY WELL AND WAS REALLY A CHALLENGE FOR US TO BE ABLE TO DECIDE WHICH ONES TO MOVE FORWARD AND WHICH ONES NOT TO.

AND SO THE DECISION WAS TAKEN AT THE LAST APPLICATION REVIEW SUBCOMMITTEE TO APPROVE THOSE PROGRAMS CONTINGENT ON THE LARGER BOARD APPROVING ADDITIONAL FUNDING TO PAY FOR THOSE PROJECTS, WHICH IS ABOUT $8 MILLION. THAT WOULD LEAD -- I DON'T THINK THERE WAS A PLAN ANYWAY TO DO ANOTHER DISCOVERY ROUND. THIS WAS A QUEST ROUND. I'M SORRY.

DR. MILLAN: SO THIS WAS THE QUEST ROUND.

MR. SHEEHY: THEY'RE VERY CLOSE ACTUALLY IN HOW THEY STACK UP. THERE'S REALLY SOME OUTSTANDING SCIENCE. IT WAS HARD FOR ME BECAUSE THERE WERE A COUPLE OF PROJECTS THAT GOT LEFT AT THE GATE THAT WERE PLURIPOTENT CELL PRODUCTS THAT ARE REALLY CENTRAL TO OUR MISSION. AND, AGAIN, THE SCIENCE WAS OUTSTANDING. SO WE MADE THAT DECISION.

I DON'T KNOW IF THERE'S -- ONE OF OUR MEMBERS WASN'T THERE WHO WAS NOT THAT KEEN ON IT. I DON'T KNOW IF HE HAS A COMMENT ON THAT, BUT I WOULD

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HOPE THAT WE COULD FUND THOSE PROJECTS BECAUSE THEY ARE VERY, VERY GOOD PROJECTS. AND IT WAS SOMEWHAT ARBITRARY HOW WE SEPARATED OUT WHICH ONES WE FUNDED AND WHICH ONES WE DIDN'T, WHICH IS KIND OF WHAT HAPPENS WHEN YOU'RE AT A POINT WHERE -- WE'RE AT A POINT, AND WE KIND OF DISCUSSED THIS IN THE PAST, WHERE WE'RE NOT GOING TO BE ABLE TO FUND ALL THE GOOD SCIENCE THAT COMES TO US UNFORTUNATELY AS WE GET TO THE END OF OUR FUNDING.

CHAIRMAN THOMAS: MR. SENATOR.

MR. TORRES: TWO THINGS. NO. 1, THANK YOU, JEFF, FOR THE WAY YOU HANDLED THAT MEETING. I THOUGHT IT WAS VERY WELL DONE AND VERY SENSITIVE TO WHAT WE NEED TO DO WITH THOSE PROJECTS.

AND SECONDLY TO MARIA FOR YOU AND YOUR STAFF'S EFFORT IN TALKING WITH THE NIH AND MOVING US IN THAT DIRECTION. I KNOW BOTH PARTIES CAME TO THE TABLE WITH EQUAL ADMIRATION, BUT IT TAKES A HERCULEAN EFFORT, AS WE ALL KNOW, TO DEAL WITH THE NIH, THOSE WHO HAVE DONE SO IN THE PAST. AND FOR US TO HAVE A PARTNERSHIP WITH THEM, I THINK, IS VERY, VERY INSTRUCTIVE.

I ALSO SPOKE YESTERDAY AFTERNOON WITH CONGRESSWOMAN BARBARA LEE, A FORMER COLLEAGUE OF MINE IN THE LEGISLATURE, AND NOW A SIGNIFICANT

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MEMBER OF THE CONGRESS, WHO REGULARLY TAKES HER MEMBERS TO UCLA TO VISIT THE SICKLE CELL LAB THERE FROM THE BLACK CONGRESSIONAL CAUCUS. AND I MIGHT ADD ALSO THAT THERE ARE A LOT OF LATINOS IN THIS COUNTRY WHO SUFFER FROM SICKLE CELL AS WELL, SO IT'S BOTH A LATINO AND AFRICAN-AMERICAN DISEASE, FOR THAT MATTER. BUT SHE IS SO HAPPY AND SUPPORTIVE OF THE 30 MILLION THAT WE'RE GOING TO HOPEFULLY VOTE AND APPROVE TODAY. AND I JUST WANTED TO PASS THAT ALONG AND ALSO TO PASS ON HER PERSONAL REGARDS TO HER HERO, BERT LUBIN.

CHAIRMAN THOMAS: ANY OTHER COMMENTS ABOUT THE BUDGET AS PRESENTED BY DR. MILLAN?

DR. MARTIN: I HAVE A SIMPLE QUESTION. THE EDUCATIONAL BUDGET, IS THAT EDUCATION OF THE TWO PROGRAMS YOU JUST DESCRIBED FOR HIGH SCHOOL AND COLLEGE?

CHAIRMAN THOMAS: YES.

DR. MILLAN: AND ALPHA CLINICS. WE HAVE AN ANNUAL SYMPOSIUM THAT THE ALPHA CLINICS HOSTS EVERY YEAR.

DR. MARTIN: JUST A COMMENT ON THOSE EDUCATIONAL PROGRAMS. ONE OF THE WAYS TO CONVINCE THE POPULATION, THE ADULTS THAT VOTE, THAT MANY YEARS AGO THAT BIOTECHNOLOGY WASN'T DANGEROUS AND

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THEY SHOULD NOT BE AFRAID OF IT, WE AT GENENTECH FUNDED PROGRAMS FOR HIGH SCHOOLS TO ACTUALLY SUPPORT LABORATORY ACTIVITY FOR HIGH SCHOOL STUDENTS.

AND THE IDEA WAS THAT WHEN THEIR PARENTS WERE LISTENING TO NEGATIVE THINGS IN THE MEDIA ABOUT FRANKENSTEIN SCIENCE, ET CETERA, THESE KIDS COULD SPEAK UP AND SAY, WAIT A MINUTE. I DID THAT TODAY. IT'S NOT SCARY. I KNOW ABOUT IT. AND THAT WAS AN EARLY ATTEMPT AT GENENTECH, AND I PAID FOR IT OUT OF AN R&D BUDGET. WE STARTED WITH SAN FRANCISCO STATE TRAINING, ET CETERA, OF HIGH SCHOOL TEACHERS, AND THEN WE HAD VANS THAT WENT AROUND TO THE HIGH SCHOOLS WHERE THE TEACHERS HAD BEEN TRAINED. AND IT HAD AN ENORMOUS IMPACT THAT WAS DIFFICULT TO MEASURE. BUT I THINK THIS IS ANOTHER SITUATION IN WHICH, WITHOUT VIOLATING THE ISSUE OF PROMOTING SOMETHING, IF WE ARE EDUCATING THE YOUNGSTERS ABOUT THE VALUE OF STEM CELLS, I THINK IT IS PART OF THAT MOMENTUM THAT WE REALLY NEED TO GAIN WITHIN THE POPULATION. AND SO I WOULD CERTAINLY ENCOURAGE PUSHING VERY HARD THOSE PROGRAMS FOR THAT PURPOSE, IF NONE OTHER.

CHAIRMAN THOMAS: THANK YOU, DR. MARTIN. I COMPLETELY AGREE WITH YOU. SENATOR TORRES WAS INSTRUMENTAL IN PARTICULARLY THE BRIDGES PROGRAM WAY

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BACK WHEN. AND I THINK THERE'S VERY LITTLE WE CAN DO THAT'S MORE VALUABLE THAN EDUCATING. SO THANK YOU FOR YOUR COMMENTS. AND DR. SAMBRANO.

MR. TORRES: AND DR. OLSON.

CHAIRMAN THOMAS: BASICALLY EVERYBODY. CONGRATULATIONS TO EVERYBODY.

SO DO WE HEAR A MOTION TO APPROVE THE BUDGET?

MR. TORRES: MOVE IT.

DR. BURTIS: SECOND.

MR. TOCHER: IF I COULD JUST PROVIDE A BIT OF DETAIL TO YOUR MOTION, SENATOR. THERE'S SORT OF THREE PARTS ENCOMPASSED IN THIS.

MR. TORRES: THAT WAS PART OF THE MOTION.

CHAIRMAN THOMAS: MR. JUELSGAARD WILL APPROVE OF SOMETHING WITH THREE PARTS.

MR. TORRES: TO A CERTAIN LIMIT.

MR. TOCHER: THE MOTION IS TO APPROVE THE BUDGET AS PROPOSED, TO CONDITIONALLY ALLOCATE UP TO $8 MILLION FROM THE CLIN1 AND 2 PORTION OF THE BUDGET TO MAKE THAT AVAILABLE TO THE APPLICATION REVIEW SUBCOMMITTEE TO CONSIDER FOR PENDING QUEST APPLICATIONS. AND, THIRD, TO BACKFILL ANY AMOUNT THAT'S USED FROM THE 2019 RECOVERY OF FUNDS BACK INTO THE CLIN1 AND 2 BUDGET.

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MR. TORRES: THAT'S EXACTLY WHAT I MEANT.

MR. TOCHER: THANK YOU.

CHAIRMAN THOMAS: VERY ELOQUENTLY SPOKEN, MR. TOCHER.

YES. THE MOTION THAT CAME OUT OF THE SCIENCE SUBCOMMITTEE WAS ABOUT TWO PAGES LONG. SO HE DISTILLED IT NICELY INTO CONCISE PROSE.

DISCUSSION ON THE MOTION BY MEMBERS OF THE BOARD? DISCUSSION, MEMBERS, EITHER IN THE ROOM OR ON THE PHONE? HEARING NONE, MARIA, WILL YOU PLEASE CALL THE ROLL? EXCUSE ME. PUBLIC COMMENT. MY FAULT. MY BAD. DON REED.

MR. REED: I JUST WANT TO MAKE SURE I UNDERSTAND. DOES THAT MEAN THAT THERE WERE FIVE OTHER PROJECTS THAT WERE SUGGESTED IN THE DISCOVERY, THAT THEIR FUNDING IS BEING INCLUDED IN THIS?

MR. TOCHER: NO.

CHAIRMAN THOMAS: WELL, IT IS TEEING THAT UP. THE ACTUAL VOTE ON THOSE PROJECTS IS LATER IN THE AGENDA.

MR. REED: THANK YOU.

CHAIRMAN THOMAS: OTHER PUBLIC COMMENTS ON THE BUDGET? OKAY. MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: AND FOR REFERENCE, THIS

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IS AGENDA ITEM NO. 8.

GEORGE BLUMENTHAL.

DR. BLUMENTHAL: YES.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: YES.

MS. BONNEVILLE: DEBORAH DEAS. DAVID BRENNER. ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: LINDA MALKAS.

DR. MALKAS: YES.

MS. BONNEVILLE: BERT LUBIN.

DR. LUBIN: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA. FRANCISCO

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PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT. AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: SUZANNE SANDMEYER.

DR. SANDMEYER: YES.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES.

MS. BONNEVILLE: OSWALD STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU, MARIA. ON TO ITEM NO. 9, UPDATE -- WE'VE ALREADY HEARD A BIT ABOUT THIS, BUT, MARIA, DO YOU WANT TO TALK MORE -- UPDATE ON THE FUNDING PROGRAM WITH NHLBI AND CIRM RE SICKLE CELL?

DR. MILLAN: I JUST WANT TO INTRODUCE GABE THOMPSON, WHO WILL BE DESCRIBING THE MOU WITH THE

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NHLBI. JUST BY WAY OF BACKGROUND, SICKLE CELL DISEASE AFFECTS APPROXIMATELY 100,000 AMERICANS, BUT IT AFFECTS MILLIONS WORLDWIDE. SO ALTHOUGH IT'S A RARE DISEASE IN THE U.S., IT'S NOT SO RARE IN OTHER PARTS OF THE WORLD. IT'S VERY IMPORTANT.

ANOTHER THING ABOUT IT IS THAT IT AFFLICTS THE YOUNG. IT LEADS TO EXTENSIVE HOSPITALIZATION AND MORBIDITY AND RESULTS IN EARLY DEATH AND WITH LIFE SPANS IN THE 40S TO 50S. SO THIS IS A VERY IMPORTANT DISEASE TO GO AFTER, AND WE'RE VERY PLEASED FOR THE OPPORTUNITY TO DO SO. GABE THOMPSON.

MR. THOMPSON: MEMBERS OF THE BOARD, CIRM TEAM, AND MEMBERS OF THE PUBLIC, I'M GABRIEL THOMPSON, VICE PRESIDENT OF GRANTS AND OPERATIONS AT CIRM. AS MARIA MENTIONED, I'M GOING TO BRIEFLY DESCRIBE THE COFUNDING INITIATIVE THAT WE HAVE WITH NHLBI FOR THE CURE OF SICKLE CELL PROGRAM.

SO AS YOU MAY HAVE KNOWN, WE SIGNED AN MOU AT THE END OF JUNE WITH THE NHLBI THAT OUTLINES THE COFUNDING INITIATIVE WHOSE PURPOSE IS REALLY TO ACCELERATE THE IMPLEMENTATION OF ACCESSIBLE CURES FOR SICKLE CELL DISEASE WITHIN FIVE TO TEN YEARS.

SO HIGHLIGHTING ACCELERATE THERE BECAUSE THAT REALLY CREATES THE MOTIVATION THAT NHLBI HAD TO

PARTNER WITH US. AND SO THERE'S AN ALIGNMENT OF PURPOSE HERE.

JUST A FEW HIGHLIGHTS OF THE PROGRAM. SO WHAT NHLBI WILL BE DOING IS PROVIDING CIRM FUNDS IN OUR CLINICAL STAGE PROGRAM. SO THAT INCLUDES THE CLIN1 IND-ENABLING PROJECTS, THE CLIN2 CLINICAL TRIAL PROJECTS, AND THE CLIN3 ACCELERATING SUPPLEMENTAL ACTIVITY PROGRAM.

SO UNLIKE SOME OTHER COFUNDING INITIATIVES THIS AGENCY HAS HAD IN THE PAST, THIS ONE IS UNIQUE IN THAT THE FUNDS WILL BE PROVIDED TO CIRM AND THE APPLICANTS WILL HAVE ONE PLACE TO GO. THEY WILL APPLY TO CIRM VIA CIRM'S APPLICATION PROCESS AND UNDER OUR PROGRAM ANNOUNCEMENTS. THE APPLICATION WILL GO UNDER ONE SCIENTIFIC REVIEW, WHICH IS OUR GRANTS WORKING GROUP. AND THEN IF THE APPLICATION IS SUCCESSFUL, IT WOULD BE MANAGED BY CIRM, ONE AWARD TO BE MANAGED BY CIRM WITH US SHARING INFORMATION AND MONITORING OF THOSE PROJECTS WITH THE NHLBI.

SO A LOT OF BENEFITS TO THE APPLICANT WHO DOESN'T HAVE TO APPLY TO TWO DIFFERENT AGENCIES, GO UNDER TWO DIFFERENT SCIENTIFIC REVIEWS. OBVIOUSLY IT PROVIDES LEVERAGED FUNDING FOR THIS AGENCY SO THAT WE CAN FUND MORE PROJECTS THAN WE COULD IF WE

WERE FUNDING ALONE. AND THEN THERE IS A DATA SHARING ELEMENT AS WELL IN THE INITIATIVE THAT IS BEING WORKED OUT THAT WILL HELP ACTUALLY MOVE THIS WHOLE FIELD FORWARD.

SO A COUPLE CHANGES THAT WE NEED TO MAKE IN ORDER TO SUPPORT THIS INITIATIVE. SO I WANT TO FIRST SAY THAT WE ATTEMPTED TO TRY TO KEEP THIS AS SIMPLE AS POSSIBLE AND TO MAKE AS LITTLE CHANGES TO OUR CLINICAL PROGRAM TO SUPPORT THIS INITIATIVE, BUT HERE ARE THE FEW ITEMS WE DO WANT TO CHANGE THAT WILL ALSO BE HIGHLIGHTED BY MY COLLEAGUE GIL SAMBRANO LATER IN THE CONCEPT PLAN CHANGES.

BUT TO GO OVER THESE, WE OBVIOUSLY HAVE TO INFORM APPLICANTS THAT THEIR APPLICATION MATERIALS WILL BE SHARED WITH NHLBI REPRESENTATIVES. THE COFUNDED AWARDEES ARE GOING TO BE REQUIRED TO COMPLY WITH NHLBI'S DATA SAFETY AND MONITORING AS WELL AS THEIR DATA SHARING POLICIES.

THIS INITIATIVE IS UNIQUE IN THAT IT ALSO WILL ALLOW NON-CALIFORNIA ORGANIZATIONS TO APPLY WHO ARE REQUESTING THEIR CIRM UNALLOWABLE COSTS TO BE COVERED BY NHLBI FUNDS. NORMALLY WE WOULD ASK THOSE FOLKS TO COME WITH THEIR OWN FUNDING TO COVER THE THINGS CIRM CAN'T FUND; BUT UNDER THIS INITIATIVE, BECAUSE WE HAVE THE NHLBI DOLLARS, WE WILL ALLOW

THESE FOLKS TO KIND OF CONCURRENTLY APPLY THOSE NHLBI FUNDS TO THE THINGS CIRM CAN'T FUND.

AND THEN FINALLY, WE WILL REQUIRE ALL SICKLE CELL APPLICATIONS TO SUBMIT UNDER THIS REVISED PROGRAM.

AS FAR AS THE PROCESS, SO, AGAIN, WE TRY TO MAKE SURE THAT WE DIDN'T CHANGE OUR PROCESS TOO MUCH. HERE, AND GIVEN THAT IT IS AN ACCELERATING PROCESS, AND WE ARE GOING TO MAINTAIN THE PROCESSING TIMES TO PROCESS CLIN APPLICATIONS IN AS LITTLE AS 80 TO 180 DAYS, APPLICATIONS COME IN UNDER THE PROGRAM AND UNDERGO THE NORMAL ELIGIBILITY CHECK IN THE FIRST MONTH, AND NHLBI WILL BE GIVEN ACCESS TO THOSE APPLICATIONS VIA OUR GRANTS MANAGEMENT SYSTEM. IF THE APPLICATIONS PASS ELIGIBILITY, THEY'LL GO TO THE GWG PEER REVIEW GROUP, IN WHICH NHLBI CAN PARTICIPATE IN THAT MEETING, AND THEN, MOST IMPORTANTLY, THE ONLY NEW STEP HERE WOULD BE THAT NHLBI WITHIN TEN DAYS OF OUR GRANTS WORKING GROUP WOULD MAKE A FUNDING DECISION THAT INCLUDED WHETHER THEY WANT TO FUND AND AT WHAT LEVEL WITHIN TEN DAYS OF THE GRANTS WORKING GROUP. SO THAT DECISION, ALONG WITH THE RECOMMENDATIONS FROM THE GRANTS WORKING GROUP, WILL COME TO THIS BOARD OR THE APPLICATION REVIEW SUBCOMMITTEE TO MAKE A FINAL

FUNDING DECISION.

AND SO WE THINK WE CAN MAINTAIN OUR PROCESSING TIMES AND STILL KEEP THE TRAINS MOVING.

FINALLY, ON THE AWARD MANAGEMENT SIDE, JUST A COUPLE THINGS TO MENTION IS THE NHLBI FUNDS ARE COMING TO CIRM VIA WHAT WE'RE LEARNING IS CALLED THE OTHER TRANSACTIONAL AUTHORITY IN FEDERAL GOVERNMENT PARLANCE WHICH ALLOWS THE FUNDS TO COME TO US WITHOUT THE NORMAL NIH GRANTS POLICY REQUIREMENTS AND REGULATIONS, AND SO THE FUNDS WILL GENERALLY BE REGULATED BY CIRM REGULATIONS.

CIRM WILL ISSUE A SINGLE NOTICE OF AWARD FOR BOTH THE CIRM AND NHLBI FUNDS, WILL UNDERGO OUR NORMAL PROGRESS AND FINANCIAL REPORTING REQUIREMENTS, AND SHARE THAT INFORMATION WITH NHLBI AGAIN VIA OUR GRANTS MANAGEMENT SYSTEM. NHLBI REPRESENTATIVES WILL BE APPOINTED TO THE CLINICAL ADVISORY PANELS THAT CIRM SETS UP IN ORDER TO HELP US WITH EXPERTISE AND HELP PROJECTS DEALING WITH BOTTLENECKS AND HELPING THEM MOVE FORWARD. AND THEN, IMPORTANTLY, CIRM RETAINS THE ABILITY TO SUSPEND OR TERMINATE THE AWARD. WE WOULD OBVIOUSLY WORK WITH NHLBI IF WE NEEDED TO GO DOWN THAT ROAD, BUT CIRM WILL RETAIN THAT ABILITY.

AND SO THAT KIND OF PRESENTS THE OVERVIEW.

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WE ARE WORKING TOWARD BRINGING THIS PROGRAM ONLINE BY THE END OF NOVEMBER AND HOPEFULLY STARTING TO ACCEPT APPLICATIONS STARTING DECEMBER 31ST. SO THAT IS MY OVERVIEW. SO I'LL TAKE ANY QUESTIONS YOU HAVE.

DR. LUBIN: SO I WANT TO CONGRATULATE YOU AGAIN, MARIA, IN GETTING THIS TOGETHER. I DON'T KNOW THAT THE NIH HAS EVER DONE THIS BEFORE. I SUSPECT THIS IS UNIQUE, AND HOPEFULLY WILL GET A LOT OF PR DISCUSSIONS RELATED TO THIS.

SO ONE OF THE THINGS THAT'S UNIQUE ABOUT CALIFORNIA IS WE STARTED NEWBORN SCREENING FOR SICKLE CELL IN THE STATE OF CALIFORNIA. WE ACTUALLY STARTED AT THE BATES HOSPITAL. AND THAT NOW IS THROUGHOUT THE UNITED STATES. SO WE KNOW AT THE BIRTH OF A CHILD WHETHER THEY HAVE SICKLE CELL. AND IF YOU'RE GOING TO DO A STEM CELL THERAPY OR A GENE THERAPY, DOING IT ON A SMALL CHILD ACTUALLY HAS BETTER OUTCOME THAN WAITING TILL LATER, TILL COMPLICATIONS ALREADY START. SO THAT'S ANOTHER INCENTIVE TO SORT OF KEEP IN MIND.

I'M CURIOUS HOW MANY APPLICATIONS YOU THINK YOU'RE GOING TO GET A YEAR OR SEVERAL MONTHS, OR IF IT'S EVERYONE IN THE UNITED STATES IS APPLYING TO NIH? ARE YOU ANTICIPATING HUNDREDS? ARE YOU

ANTICIPATING TEN?

DR. MILLAN: SO WHAT WE -- AS PART OF THIS EXERCISE, THE TEAM WENT THROUGH AN EXERCISE WITH THE NHLBI IN TERMS OF JUST CHARTING OUT WHO WE KNOW ARE PROSPECTS BASED ON WHO'S ALREADY IN OUR PIPELINE AND POTENTIAL --

DR. LUBIN: THAT'S FOR CALIFORNIA.

DR. MILLAN: NO, JUST IN GENERAL. THERE ARE NOT A HUGE AMOUNT OF FOLKS OUT THERE, BUT WE BELIEVE THAT IN TERMS OF THOSE WHO MAY BE READY TO COME IN FOR THE CLINICAL STAGE PROGRAMS, APPROXIMATELY SIX OR SO IN THE NEAR TERM. SO WHEN WE PROPOSE THIS BUDGET SET ASIDE, IT IS IN ANTICIPATION OF THAT SHARING THE COST FOR APPROXIMATELY SIX PROGRAMS OVER THE SPAN OF A YEAR, YEAR AND A HALF, OR TWO.

IT'S POSSIBLE THAT THERE MAY BE MORE THAT START TO COME OUT OF THE WOODWORK ONCE WE GET THIS ROLLING, BUT WE BELIEVE WE HAVE CAPTURED THE VISIBLE ONES. AND THESE ARE THE ONES WHERE WE'VE ALREADY HAD DISCUSSIONS WITH THEM, HAVE ALREADY BEEN WORKING WITH THEM EITHER BECAUSE THEY'RE ACTIVE GRANTEES READY TO MOVE TO THE NEXT STAGE, OR THEY'RE INTERESTED PARTIES WHO SEE A VALUE IN PARTNERING WITH CIRM AND NOW WITH CIRM AND THE NIH.

DR. LUBIN: I WAS THINKING OF PEOPLE THAT ARE NOT IN CALIFORNIA THAT ARE DOING STEM CELL RESEARCH RELATED TO SICKLE CELL. DOES THAT INCLUDE THAT?

DR. MILLAN: THAT INCLUDES THAT. THERE MAY BE MORE, BUT WE WERE JUST LOOKING AT NEAR TERM BECAUSE WE WANTED TO MAKE SURE THAT WHAT WE DID IS PLAN TO HAVE A BUDGET IN PLACE SO THAT ONCE THEY COME IN, WE'RE READY TO GO BECAUSE THAT'S THE WHOLE POINT OF ACCELERATION. AND IT WOULD BE A SHAME TO HOLD THINGS UP AND HAVE PEOPLE PLAN FOR IT AND NOT HAVE IT READY TO GO.

DR. LUBIN: THAT'S WONDERFUL. THE OTHER THING IS THE NIH, AS YOU KNOW, MANY OF US KNOW, SENDS OUT ANNOUNCEMENTS OF NEW PROGRAMS, OF NEW ACTIVITIES. DID THEY PREPARE A PUBLIC DOCUMENT THAT'S GOING TO GO OUT ABOUT THIS NEW RELATIONSHIP TO ALL INVESTIGATORS INTERESTED IN THIS AREA AND NOT TO WORRY THAT IT'S GOING TO SLOW THINGS DOWN. IN FACT, IT'S GOING TO MAKE THINGS FASTER. I THINK IT'S A GREAT OPPORTUNITY EVEN WITH OUR -- DON'T WANT TO USE THE WORD "CAMPAIGN," BUT REALLY EVEN AS PART OF THAT. I THINK IT'S A GREAT OPPORTUNITY.

DR. MILLAN: OUR COMMUNICATIONS GROUP HAD WORKED WITH NIH ON A JOINT PRESS RELEASE. IN

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ADDITION, OUR TEAMS WILL WORK TOGETHER IN TERMS OF COORDINATING THE PROGRAM ANNOUNCEMENT SO THAT THEY CAN SEAMLESSLY BE DIVERTED INTO THIS PROGRAM ANNOUNCEMENT IF THEY'RE CELL AND GENE THERAPY BECAUSE THE CURE SICKLE CELL PROGRAM ENCOMPASSES OTHER APPROACHES AS WELL, BUT WE ARE THE SPECIALTY SHOP FOR CELL/GENE THERAPY. SO WHAT WE'RE WORKING WITH THEM ON IS STREAMLINING THAT TO HANDLE IT THROUGH THIS MECHANISM.

DR. LUBIN: NICE FOR THE BOARD. IT WOULD BE GREAT IF THE BOARD GETS A CHANCE TO SEE WHATEVER THAT PR DOCUMENT IS.

DR. MILLAN: WE'LL DEFINITELY SEND IT ALL AROUND.

DR. LUBIN: THANKS.

DR. PRIETO: YES, I HAD A QUESTION. IF THE FEDERAL GOVERNMENT WERE TO SUCCEED IN IMPOSING NEW RESTRICTIONS ON STEM CELL RESEARCH FUNDING OR CUTS TO NIH FUNDING, COULD THAT IMPACT THIS INITIATIVE?

MR. THOMPSON: I DON'T KNOW THE ANSWER TO THAT. WE HAVE ASKED THEM IF THOSE THINGS WOULD -- ANY KIND OF DECISION LIKE THAT WOULD IMPACT THIS PROGRAM, AND WE HAVEN'T BEEN TOLD THAT IT WOULD. BUT OBVIOUSLY IT DEPENDS ON WHAT WOULD HAPPEN IF

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THERE'S NEW LEGISLATION.

DR. MILLAN: WE HAD CONVERSATIONS ABOUT THE BUDGET. THIS IS A FIVE-YEAR MOU. THEIR BUDGET, THE NHLBI BUDGET IS A LITTLE BIT OVER $3 BILLION FOR THIS. AND I THINK ONE OF THE ADVANTAGES OF THE OTHER TRANSACTION AUTHORITY IS THE WAY THEY CAN BUDGET AND MAKE SURE THAT THIS IS SOMETHING THAT WILL BE ACCESSIBLE FOR THIS INITIATIVE. BUT WE DON'T HAVE ANY INDICATIONS FROM THEM THAT THEY THINK THIS IS GOING TO BE AT RISK GIVEN THE TIME FRAME THAT WE HAVE SET OUT AND GIVEN THE BUDGET THAT THEY HAVE ACCESS TO. SO...

THERE'S NEVER ANY GUARANTEES. SOMEBODY COULD JUST CUT OFF ALL FUNDING TOMORROW. BUT SHORT OF THAT, I THINK THEY FEEL PRETTY CONFIDENT THAT THEY'LL BE ABLE TO EXECUTE ON THIS.

CHAIRMAN THOMAS: I WOULD ADD TO THAT. I BELIEVE THE SUGGESTIONS THAT HAVE BEEN FLOATED AS TO WHAT SORT OF MEASURES THE ADMINISTRATION WOULD TAKE WITH RESPECT TO NIH MAY NOT IMPACT THE SUBJECT MATTER OF THIS PARTICULAR TYPE OF RESEARCH. I THINK IT WOULD BE LIMITED, ALTHOUGH WHO KNOWS. AS DR. MILLAN SAYS, YOU JUST DON'T KNOW WHAT'S GOING TO HAPPEN.

DR. DULIEGE: WHAT WOULD YOU ANTICIPATE

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WILL BE THE AMOUNT OF COFUNDING THAT WILL COME FROM THE NIH AS PART OF THIS PROGRAM?

DR. MILLAN: SO IN ROUGH FIGURES, WHAT WE'RE HOPING FOR IS THEY SUPPORT CLOSE TO AT LEAST HALF OF WHAT THE CIRM EXPOSURE WOULD BE. AND WHAT WE'RE WORKING ON RIGHT NOW IS KIND OF FINALIZING WHAT THOSE CONTRACTS WILL LOOK LIKE. SO THEY LOOKED AT THAT AND THOUGHT IT WAS REASONABLE. BUT, OF COURSE, THEY COULDN'T REALLY -- WE'RE WORKING A PRETTY SHORT TIME FRAME HERE. THEY COULDN'T REALLY COMMIT TO A DOLLAR AMOUNT AT THIS POINT, BUT THE SPIRIT OF IT IS THAT THEY KNOW THEY'LL SHARE WITH THE CIRM COSTS; BUT, IN ADDITION, THEY WOULD ALSO PROBABLY COVER SOME OF THE APPLICANT'S ADDITIONAL COSTS THAT CIRM WOULDN'T FUND. SO, FOR INSTANCE, THEIR EXPOSURE COULD BE A LOT HIGHER FOR NON-CALIFORNIA APPLICANTS BECAUSE THEY'D SHARE IN OUR COSTS, WHICH FOR THE CALIFORNIA APPLICANTS WOULD BE MORE, BUT THEN IN ADDITION, THEY'D COVER THE NON-CALIFORNIA COSTS.

DR. MALKAS: MARIA, ONE OTHER QUESTION. THE PROGRAM HERE IS VERY MILESTONE DRIVEN, AND YOU'RE NOT AFRAID TO PULL BACK FUNDS. IS THAT GOING TO BE INCORPORATED INTO THAT PROGRAM, INTO THIS PROGRAM AS WELL?

MR. THOMPSON: YES, ABSOLUTELY.

DR. MILLAN: IN FACT, THE WHOLE SYSTEM, THE WHOLE FUNDING MODEL IS SOMETHING THAT ATTRACTED THEM. AND ALSO, BY THE WAY, JUST KUDOS TO OUR I.T. DEPARTMENT, GMS, THE GRANT MANAGEMENT SYSTEM. THEY SAID IT WOULD TAKE THEM TEN YEARS TO BUILD SOMETHING LIKE THAT TO EVEN GET THIS STARTED TO SUPPORT SUCH AN ACCELERATION MODEL. AND MEANWHILE WE HAVE A FIVE- TO TEN-YEAR CURE INITIATIVE. THAT WAS ANOTHER KIND OF TECHNICAL ASPECT. AND JUST THE WAY THAT SCOTT TOCHER, GABE, AND GIL WERE THERE IN TERMS OF HOW THEY CAN ACCESS THE INFORMATION AND STILL BE IN COMPLIANCE, THAT WAS VERY ATTRACTIVE TO THEM.

DR. JUELSGAARD: A HYPOTHETICAL. SO AS I UNDERSTAND IT, THESE PROGRAMS, PROPOSALS, ALLOW FOR BOTH EX-CALIFORNIA AS WELL AS WITHIN CALIFORNIA PARTICIPATION. IT'S JUST THAT CIRM FUNDING WOULD BE LIMITED TO THE CALIFORNIA ASPECT OF IT. SO WOULD IT BE POSSIBLE, FOR EXAMPLE, TO HAVE A PROJECT IN WHICH 80 PERCENT OF THE WORK IS BEING DONE OUTSIDE OF THE STATE OF CALIFORNIA AND 20 PERCENT WITHIN THE STATE AND WE JUST FUND THAT 20 PERCENT? WOULD THAT BE ALLOWABLE? BECAUSE THEN IT GOES TO THE QUESTION OF POST GRANT MANAGEMENT BECAUSE NOW ALL OF A SUDDEN THE POST GRANT MANAGEMENT ASPECT OF IT OUTSIDE THE

STATE OF CALIFORNIA BEING FUNDED, I GATHER, IN THIS CASE BY THE NHLBI. SO HOW DO WE ENVISION MANAGING SOMETHING WHERE SO MUCH OF THE WORK IS BEING DONE OUTSIDE THE STATE, BUT WE'RE RESPONSIBLE FOR MANAGING IT?

DR. MILLAN: WE ACTUALLY HAVE GRANTEES OUTSIDE OF THE STATE EVEN NOW. SO WE WOULD MANAGE IT IN THE SAME WAY, AND THEY WOULD DEFER TO OUR MANAGEMENT OF THIS AND BE IN CONTACT WITH US BECAUSE THE WHOLE -- THEY'RE ACCEPTING THE WHOLE PACKAGE, AND THEY UNDERSTAND, NOT ONLY IS IT THE DUE DILIGENCE OR THEIR REVIEW PROCESS, BUT IT'S OUR MILESTONE-DRIVEN MANAGEMENT OF THE AWARD, OUR CLINICAL ADVISORY PANEL, INFRASTRUCTURE PROGRAMS WE COULD PUT TO IT. THEY ALSO HAVE INFRASTRUCTURE PROGRAMS THAT THEY'D LIKE TO OFFER TO THIS, BUT THEY SEE THE VALUE IN THE WHOLE PACKAGE. SO THE IDEA IS THAT CIRM WOULD TAKE THE LEAD IN MANAGING ANY AWARD REGARDLESS OF PERCENTAGE WITHIN CALIFORNIA.

DR. JUELSGAARD: SO JUST TO FOLLOW UP ON THAT, DR. MILLAN. SO IMAGINE THAT 2020 COMES AND GOES AND THERE'S NO ADDITIONAL FUNDING, BUT WE HAVE A FIVE-YEAR COMMITMENT UNDER THIS PROGRAM. WE'VE GOT THEM THE FUNDING ON THE ADMINISTRATIVE SIDE TO HANDLE THAT GRANTS MANAGEMENT OUTSIDE OF THE STATE

OF CALIFORNIA IN THIS CIRCUMSTANCE THAT'S INCLUDED IN THE FUNDING THAT WE'RE TALKING ABOUT, NOT NECESSARILY THE 30 MILLION?

DR. MILLAN: YES. WE'RE NEGOTIATING ADMINISTRATIVE COVERAGE OF ADMINISTRATIVE COSTS ASSOCIATED WITH IMPLEMENTING THIS. AND THEY ARE AWARE OF THE 2020 TIMELINE, AND WE'RE PUTTING IN PLACE PLANS THAT, SHOULD IT EXCEED EVEN OUR ADMINISTRATIVE PLANS BEYOND 2020, THAT THEY WOULD BE ABLE TO PICK IT UP AND CARRY IT FORWARD BEYOND THAT.

DR. JUELSGAARD: GREAT. JUST ONE LAST QUESTION. WHAT IS THE EXACT POINT AT WHICH THEY HAVE TO BE IN ORDER FOR US TO CONSIDER THEIR PROGRAM? I KNOW ON THE SLIDE IT SAYS PRE-IND MEETING OR EQUIVALENT. BUT IF YOU HAD TO PICK A POINT, DESCRIBE A POINT A PROGRAM NOW FALLS INTO BEING CONSIDERED AS OPPOSED TO IT'S TOO EARLY TO BE CONSIDERED? WHAT IS THAT POINT?

DR. MILLAN: IT'S EQUIVALENT TO OUR CLIN1 WHICH IS THAT THEY HAVE HAD A PRE-IND MEETING AND THEY MEET ELIGIBILITY TO COME IN FOR A CLIN1.

DR. MARTIN: WHO IS THE SIGNING AUTHORITY ON THIS, MARIA? IS IT THE COUNCIL OR THE DIRECTOR?

DR. MILLAN: IT'S GARY GIBBONS, WHO IS THE HEAD OF NHLBI, THE INSTITUTE DIRECTOR.

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DR. SANDMEYER: I'M JUST CURIOUS. IN THE LONG TERM, DOES NIH ENVISION THAT THIS IS A MODEL THAT THEN THEY WOULD ADOPT MORE BROADLY FOR GENE THERAPY TRIALS? OR DO WE IMAGINE THAT WE WOULD EXPAND THIS COOPERATIVE EFFORT WITH THE FEDERAL GOVERNMENT?

DR. MILLAN: SO THE GENESIS OF THIS WAS LAST JUNE A TEAM OF US WENT -- FRANCES COLLINS, THE HEAD OF NIH, INVITED US TO COME TO THE NIH TO DESCRIBE HOW WE FUNDED AND MANAGED PROGRAMS. AND WE HAD SUCH AN INTEREST LEVEL, THAT WE MET WITH AT LEAST 13 INSTITUTE HEADS. WE ACTUALLY HAD TO PROLONG OUR TIME THERE IN ORDER TO MEET WITH THEM ALL BECAUSE THEY WERE VERY INTERESTED ESPECIALLY IN THE ARENA OF REGENERATIVE MEDICINE, STEM CELL, AND GENE THERAPY. NHLBI, BECAUSE OF THIS CURE SICKLE CELL, WAS THE FIRST ONE OUT THE GATE. THERE HAVE BEEN OTHER INSTITUTES WHO HAVE EXPRESSED INTEREST AND THINKING ABOUT HOW WE COULD WORK TOGETHER IN A SIMILAR WAY. SO WE VIEW THIS AS AN IMPORTANT PROGRAM ON ITS OWN, BUT ALSO POTENTIALLY A NICE PILOT FOR POTENTIAL COFUNDING OPPORTUNITIES IN THE FUTURE.

DR. SANDMEYER: THANK YOU. I'M ALSO ECHOING EARLIER COMMENTS. IT'S EXCITING HOW IT

COULD EXPAND OUR PROFILE HERE IN CALIFORNIA.

DR. MILLAN: THANK YOU.

CHAIRMAN THOMAS: ANY OTHER COMMENTS FOR MR. THOMPSON?

MR. SHEEHY: JUST A COUPLE QUESTIONS. ONE IS TO CONFIRM. SO THE REVIEW WILL BE SEPARATE FROM THE OTHER REVIEW. SO WE'RE NOT PUTTING -- WE'RE NOT REVIEWING -- SAY, WE DO OUR MONTHLY CLIN REVIEW. THIS WOULDN'T BE IN WITH THE SAME BUCKET OF APPLICATIONS. SO THEY WOULD BE IN A SEPARATE BUCKET PERHAPS WITHIN THE SAME REVIEW, BUT JUST CLARIFYING THAT POINT.

DR. MILLAN: I'M GOING TO DEFER TO GIL SAMBRANO. MAYBE HE CAN RESPOND TO IT AT THIS POINT.

MR. SHEEHY: THE REASON IS THAT THERE'S ALWAYS SOMETHING COMPETITIVE ABOUT THIS. AND THIS PARTICULAR INITIATIVE, I THINK APPLES SHOULD BE COMPARED TO APPLES AND NOT NECESSARILY BLEND IN WITH APPLES THE OTHER THINGS THAT WE MAY BE DOING SO THAT THAT'S CLEAR TO THE REVIEWERS, THAT WE'RE REALLY FOCUSED ON SICKLE CELL IN THIS PARTICULAR BUCKET. EVEN IF IT'S WITHIN THE SAME REVIEW, IT COULD HAVE PHASE I AND PHASE 2 OR SOMETHING ALONG THOSE LINES.

DR. SAMBRANO: SO THE INTENT IS TO HAVE IT WITHIN THE SAME REVIEW. THAT MAKES IT EASY TO JUST

INCORPORATE INTO OUR 9 A.M. CYCLES PER YEAR. BUT, YES, WE WILL HAVE TO NOTE THE DIFFERENCE IN THIS PROGRAM AND HOW IT IS UNIQUE AND DIFFERENT FROM THE OTHER APPLICATIONS THAT MAY BE CONSIDERED. WE DON'T NECESSARILY EXPECT TO GET ALL SICKLE CELL APPLICATIONS ALL AT ONCE WHERE WE WOULD BE LOOKING AT THEM NECESSARILY TOGETHER. WE MIGHT IN SOME CASES, BUT IN GENERAL THEY'RE GOING TO TRICKLE IN AS THEY ARE READY TO COME IN.

MR. SHEEHY: I'M JUST REALLY FOCUSED ON THE POINT OF HAVING CLEAR DELINEATION BECAUSE THERE IS ALWAYS, IN MY EXPERIENCE IN BEING IN THE REVIEWS, AND ONE OF THE THINGS I'VE ALWAYS FOUND FRUSTRATING, BY THE WAY, BECAUSE OUR POLICY HAS ALWAYS BEEN EACH REVIEW -- THE PROJECTS ARE NOT IN COMPETITION WITH EACH OTHER. INEVITABLY THAT'S HOW PEOPLE VIEW THEM. SO FOR THIS PARTICULAR INITIATIVE, IF WE CAN CLEARLY DELINEATE THAT WE HAVE SET ASIDE FUNDS FOR THIS, THAT THIS SHOULD BE REVIEWED SEPARATELY FROM THE OTHER APPLICATIONS, AND SHOULD NOT BE VIEWED IN COMPETITION WITH OTHER APPLICATIONS THAT MAY BE COMING IN.

DR. SAMBRANO: WE WILL MAKE AN EFFORT TO DO THAT.

MR. SHEEHY: MY OTHER QUESTION IS, SO

WE'RE ON TRACK, WHAT DID YOU SAY, ABOUT 43 OF THE PROJECTED 50 CLINICAL TRIALS, DOES THAT INCLUDE THIS INITIATIVE, OR WOULD THIS INITIATIVE BE ADDITIVE TO THAT?

DR. MILLAN: THAT INCLUDES THIS INITIATIVE.

MR. SHEEHY: GREAT. THANK YOU.

CHAIRMAN THOMAS: ANY OTHER COMMENTS FOR MR. THOMPSON, DR. SAMBRANO, DR. MILLAN? COMMENTS MEMBERS OF THE PUBLIC?

DR. CHIU: ARLENE CHIU FROM THE CITY OF HOPE. I REALLY AM EXCITED ABOUT THIS JOINT INITIATIVE. I THINK IT'S A GROUNDBREAKING EFFORT, AND I CONGRATULATE CIRM FOR PULLING IT OFF.

JUST SOME MINOR DETAILS ABOUT HOW THIS IS ROLLED OUT. I HEAR YOU MENTIONED THAT YOU HOPE TO GET APPLICATIONS OR ROLL IT OUT BY THE END OF THE YEAR. THAT MEANS NIH WILL HAVE TO HUSTLE TO GET THE FOA OUT ASAP. AND IF I UNDERSTAND CORRECTLY, YOU WILL MAINTAIN YOUR MONTHLY CLIN ACCEPTANCE SO THAT AS THEY TRICKLE IN, AND ESPECIALLY THOSE THAT ARE FROM OUTSIDE OF CALIFORNIA WHO ARE NOT USED TO THE SYSTEM, THERE MAY BE UPS AND DOWNS IN THE KINDS THAT YOU GET; BUT IF I UNDERSTAND CORRECTLY, THIS ACCEPTANCE OF THESE APPLICATIONS WILL END BY THE END

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OF 2019, OR DO YOU ANTICIPATE MORE PROPOSALS COMING IN IN 2020?

DR. MILLAN: THERE'S NO END DATE. WE'RE ASKING FOR THE ALLOCATION NOW SO THAT WE'RE ABLE TO FUND THEM IF THEY HAPPEN TO ALL COME IN IN 2019. IT'S ENTIRELY POSSIBLE THAT SOME OF THOSE THAT WE ANTICIPATE COMING IN WON'T MAKE IT IN UNTIL THE END OF 2019 OR 2020. SO WE'RE NOT PROPOSING AN EXPIRATION FOR THE PROPOSED 30 MILLION SET ASIDE.

DR. CHIU: BUT IT WILL END WHEN CIRM SPENDS OR COMMITS THE 30 MILLION. AND YOU'RE COMMITMENT IS LOOKED AT IN A DIFFERENT WAY THAN NIH COMMITMENT. NIH COMMITS JUST WHATEVER THEY SPEND THAT YEAR. SO THEIR COMMITMENT TO YOUR PROGRAM WILL BE SPREAD OUT OVER FIVE YEARS WITH EACH YEAR THEM PAYING IT DOWN; WHEREAS, YOU MAKE A COMMITMENT, YOU LOCK UP THAT FULL AMOUNT. SO YOUR 30 MILLION COULD BE QUITE QUICKLY LOCKED UP IF YOU GET REALLY WONDERFUL PROPOSALS OR IF YOU'RE EXCITED ABOUT THE EARLIER ONES, AND THEN AS OTHERS COME, THEY WILL HAVE LESS OF A CHANCE. AM I UNDERSTANDING THIS CORRECTLY?

DR. MILLAN: YES. AND THEN WHEN WE GET -- IF WE HAVE THAT GOOD PROBLEM, WHICH WE REALLY -- I THINK THAT WOULD BE A SUCCESS TO HAVE SIX EXCELLENT

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PROGRAMS COME IN AT ONCE. THEN WE'LL HAVE TO FIGURE OUT WHERE ADDITIONAL FUNDS CAN COME FROM. AND, AGAIN, WE WOULD HAVE ACCESS TO THE NIH FOR FUNDING.

SO WE'LL HAVE TO SEE WHAT HAPPENS AT THAT POINT. BUT SUFFICE IT TO SAY THAT THEY WILL RELY ON OUR PROGRAMS. SO ONE CAN ENVISION THAT IT COULD BE THAT TOWARD THE END OF THIS THAT THEY WOULD BE THE FUNDING SOURCE FOR THE MAJORITY OF THE PROGRAMS. WE'LL HAVE TO SEE HOW IT GOES.

DR. CHIU: FINALLY, OF COURSE, THE DIRECTOR OF NHLBI MAKES ALL FINAL FUNDING DECISIONS AS WITH ANY INSTITUTE AT NIH. BUT OFTENTIMES THEY PRESENT BEFORE COUNCIL AND GETS COUNCIL STAMP OF APPROVAL BEFORE THE DIRECTOR IS ALLOWED TO APPROVE FUNDING. BUT THAT ONLY MEETS THREE TIMES A YEAR. IS THAT GOING TO BE A BIT OF A PROBLEM FOR YOU?

DR. MILLAN: UNDER THE OTA ALL THAT WOULD BE REQUIRED TO MAKE THAT 10-DAY TIME FRAME DECISION IS IT WOULD GO TO THE EXECUTIVE COUNCIL WHO WOULD PROPOSE IT TO DR. GIBBONS, AND DR. GIBBONS WOULD MAKE THE FINAL DECISION.

DR. CHIU: OKAY. THANK YOU.

CHAIRMAN THOMAS: ANY OTHER COMMENTS?

OKAY. I WOULD LIKE TO ECHO EVERYTHING, MARIA, GABE, GIL, SCOTT. THIS IS OUTSTANDING, VERY

GROUNDBREAKING MOVE. AND I DO THINK, FURTHER TO DR. SANDMEYER'S QUESTION, COULD WELL PROVIDE THE TEMPLATE FOR SIMILAR SORTS OF THINGS WITH RESPECT TO OTHER CONDITIONS. AND IT'S A TREMENDOUSLY VALIDATING MOVE BY THE NIH AS FAR AS CIRM IS CONCERNED. I JUST WANT TO CONGRATULATE YOU AND THE TEAM FOR A REALLY EXCELLENT PROGRAM.

DR. MILLAN: THANK YOU VERY MUCH.

CHAIRMAN THOMAS: SO TO GIVE BETH A BIT OF A BREAK, WE'RE GOING TO TAKE TEN MINUTES HERE, AND WE'LL RESUME PROMPTLY, LOOKS LIKE, 11 O'CLOCK.

(A RECESS WAS TAKEN.)

CHAIRMAN THOMAS: WE'RE GOING TO RESUME. OKAY. WE ARE GOING TO GO TO ITEM NO. 10, CONSIDERATION OF AMENDMENTS TO THE CONCEPT PLAN FOR TRANSLATION RESEARCH PROGRAMS. DR. SAMBRANO.

DR. SAMBRANO: THANK YOU, MR. CHAIRMAN. SO I'M GOING TO PRESENT AN OVERVIEW OF THE CHANGES THAT WE'RE MAKING TO THE CONCEPTS THAT AFFECT BOTH THE TRANSLATIONAL AND CLINICAL PROGRAMS. SO I'LL GIVE YOU A LITTLE BIT OF DETAIL ON SOME OF THESE THINGS.

BIG PICTURE IS THAT THESE CONCEPT CHANGES ARE GOING TO REMOVE SMALL MOLECULES AND BIOLOGICS FROM ELIGIBILITY FOR THE TRAN AND CLIN1 PROGRAMS

EXCEPT FOR THOSE THAT ARE PREVIOUSLY FUNDED AS A PIPELINE PROJECT, MEANING THAT IS A PROGRAM THAT WE HAVE FUNDED BEFORE, AS WELL AS THE SICKLE CELL PROJECTS.

WE'RE ALSO ADDING IN VIVO GENE THERAPY TO THESE TWO PROGRAMS AND THEN ADDING REQUIREMENTS THAT GABE THOMPSON REVIEWED RELATED TO THE NHLBI/CIRM CURE SICKLE CELL JOINT INITIATIVE.

SO LET ME JUST START WITH THE TRANSLATION PROGRAM AND HOW THE CONCEPT CHANGES AFFECT THIS PROGRAM.

SO WHAT YOU'RE SEEING IS AN ILLUSTRATION OF THE TRANSLATION PROGRAM AS IT CURRENTLY EXISTS. THE TRANSLATION PROGRAM ACCEPTS STUDIES THAT HAVE DONE A PROOF OF CONCEPT SUCH AS THROUGH A DISC2 PROGRAM. AND SO THE TRAN ALLOWS THE DEVELOPMENT OF PRODUCTS THAT ARE EITHER A THERAPEUTIC, A DIAGNOSTIC, A DEVICE, OR A TOOL. THERE ARE DIFFERENT MAXIMUM TIMES AND MAXIMUM ALLOWABLE COSTS FOR EACH OF THESE DIFFERENT PRODUCT TYPES SINCE THE TRANSLATIONAL DEVELOPMENT DOES DIFFER FOR EACH OF THESE PRODUCTS. THE THERAPEUTIC IS ONE THAT WE GET THE MOST OF. OVER 95 PERCENT OF THE APPLICATIONS THAT WE GET ARE IN THE THERAPEUTIC ARENA. AND SO FOR THOSE, WE TYPICALLY ACCEPT SMALL MOLECULES OR

BIOLOGICS THAT ACT ON A STEM CELL OR A CELL THERAPY THAT INVOLVES A STEM CELL.

THE WAY WE ANTICIPATE CHANGING THIS PROGRAM IS THAT FOR AT LEAST NEXT YEAR, AND THIS IS NOT SPECIFICALLY A CONCEPT CHANGE, BUT OUR INTENT IS TO ONLY ISSUE A SOLICITATION FOR THE TRAN1 PROGRAM. THE CONCEPT CHANGE, HOWEVER, WOULD BE THAT FOR PIPELINE PROJECTS, MEANING THOSE THAT HAVE RECEIVED PREVIOUS CIRM FUNDING, THEY WOULD STILL BE ELIGIBLE UNDER THE CONVENTIONAL ELIGIBILITY CRITERIA, MEANING SMALL MOLECULES AND BIOLOGICS AS WELL AS CELL THERAPY WOULD BE ALLOWED. FOR NEW PROJECTS COMING IN, WE WANTED TO LIMIT THAT TO CELL THERAPY.

WE ARE ALSO ADDING THE IN VIVO GENE THERAPY, WHICH I WILL GET TO, BUT THE SCOPE OF NEW PROJECTS WOULD INCLUDE CELL THERAPY OR IN VIVO GENE THERAPY FOR TRAN1.

FOR THE CLINICAL PROGRAM, THAT INCLUDES OUR CLIN1, CLIN2, AND CLIN3 PROGRAMS. THIS IMPACTS JUST CLIN1 AND CLIN2 WHICH OFTEN TAKE STUDIES THAT HAVE AT LEAST A CLIN1, ACHIEVED A PRE-IND MEETING AND ARE READY TO START IND-ENABLING ACTIVITIES, OR FOR CLIN2, THOSE THAT ARE READY TO START A CLINICAL TRIAL. SO THERE ARE DIFFERENT TIMELINES AS NOTED, BUT IN GENERAL THEY BOTH HAVE ACCEPTED SMALL

MOLECULES, BIOLOGICS, CELL THERAPY, AND DEVICES INTO THE PROGRAM.

THE WAY THIS WOULD CHANGE IS THAT THE CLIN1 PROGRAM FOR PIPELINE PROJECTS WOULD STAY THE SAME. FOR NEW PROJECTS WE WOULD ACCEPT CELL THERAPY AS WELL AS IN VIVO GENE THERAPY PROJECTS ONLY. FOR THE CLIN2, THE CLINICAL TRIAL PROGRAM, THAT WOULD NOT UNDERGO ANY ADDED RESTRICTIONS, BUT ACTUALLY ADD IN VIVO GENE THERAPY AS A NEW OPTION AVAILABLE TO APPLICANTS.

SO THIS ADDITION TO THE PROGRAMS OF IN VIVO GENE THERAPY IS A NEW ELIGIBILITY CRITERIA. SO THE THING I WANT TO NOTE, JUST BECAUSE IT IS DIFFERENT FROM OTHER ELEMENTS, WE HAVE ALWAYS INCLUDED GENE THERAPY IN THE CONTEXT OF A STEM CELL. SO WHEREVER A STEM CELL IS INVOLVED, THAT HAS NOT GENERALLY BEEN AN ISSUE WHETHER IT'S A GENE THERAPY THAT TARGETS A STEM CELL OR IF IT'S, FOR EXAMPLE, A HEMATOPOIETIC STEM CELL THAT HAS BEEN GENE MODIFIED HAS NORMALLY BEEN ELIGIBLE. WHAT WE'RE TALKING ABOUT HERE IS IN VIVO GENE THERAPY PROJECTS THAT DO NOT IMPACT ON A STEM CELL OR DO NOT INVOLVE A STEM CELL IN ANY WAY.

AND SO THE WAY WE CAN DO THIS IS PROP 71 ALLOWS FOR VITAL RESEARCH OPPORTUNITIES. AND I'M

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JUST GOING TO DESCRIBE WHAT THAT DEFINITION IS. SO IT MEANS A SCIENTIFIC AND MEDICAL RESEARCH TECHNOLOGY AND/OR ANY STEM CELL RESEARCH NOT ACTUALLY FUNDED BY THE INSTITUTE UNDER, AND THEN THE REFERENCE OF WHAT WE NORMALLY FUND, WHICH PROVIDES A SUBSTANTIALLY SUPERIOR RESEARCH OPPORTUNITY VITAL TO ADVANCE MEDICAL SCIENCE AS DETERMINED BY AT LEAST A TWO-THIRDS VOTE OF A QUORUM OF THE MEMBERS OF THE GRANTS WORKING GROUP.

SO FOR PROJECTS THAT COME IN UNDER THIS GUISE, WE WILL HAVE THE GRANTS WORKING GROUP TAKE A SPECIFIC VOTE TO ENSURE THAT THEY BELIEVE THAT THIS DOES REPRESENT A VITAL RESEARCH OPPORTUNITY AS DEFINED.

AND THEN THE LAST SET OF CHANGES ARE IN SUPPORT OF THE SICKLE CELL DISEASE JOINT INITIATIVE BETWEEN CIRM AND NHLBI, MANY OF THESE ALREADY DESCRIBED BY GABE THOMPSON, CLEARLY THAT ALL SICKLE CELL DISEASE APPLICATIONS WILL BE CONSIDERED FOR JOINT FUNDING, THAT THE SICKLE CELL PROJECTS WILL BE EXEMPT FROM ANY OF THE CLIN1 THERAPEUTIC CANDIDATE RESTRICTIONS, THAT WE WILL SHARE APPLICATION MATERIALS WITH NHLBI, THAT NON-CALIFORNIA APPLICANTS MAY APPLY FOR NHLBI FUNDS TO COVER UNALLOWABLE ACTIVITIES OUTSIDE OF CALIFORNIA, AND, FINALLY, THAT

COFUNDED PROJECTS MUST ADHERE TO NHLBI POLICIES FOR DATA AND SAFETY MONITORING AS WELL AS DATA SHARING, AND THIS INCLUDES A COORDINATING CENTER FOR SICKLE CELL DATA THAT IS PART OF THE OVERALL PROGRAM THAT IS BEING PUT TOGETHER. SO THOSE ARE THE PROPOSED CHANGES.

SO ARE THERE ANY QUESTIONS? WE'RE LOOKING FOR APPROVAL OF THESE CONCEPT CHANGES FOR TRANSLATION AND CLINICAL PROGRAMS.

DR. MARTIN: DOES THIS CHANGE THE MESSAGING OF CIRM? IT'S REGENERATIVE MEDICINE, BUT IT'S NOT STEM CELL RELATED DIRECTLY?

DR. SAMBRANO: I THINK IT EXPANDS OUR CAPABILITY. SO YES. IT EXPANDS OUR CAPABILITY BEYOND STEM CELLS. BUT THE QUESTION OF WHETHER THAT MAKES SENSE OR NOT REALLY IS A DISCUSSION ITEM MAYBE FOR THIS BOARD.

DR. MARTIN: HOW DEPENDENT DO YOU THINK THE SUPPORT AMONG THE POPULATION, LEGISLATION, ET CETERA IS? HOW DEPENDENT IS IT ON THIS STEM CELL CONCEPT? IS THAT CONSIDERED TO BE RESTRICTING, WHICH IS WHERE IT ORIGINATED WHEN THERE WAS A BAN ON USE OF STEM CELLS, HUMAN STEM CELLS. I JUST DON'T KNOW WELL ENOUGH. I'VE ALWAYS THOUGHT ANY APPLICATION HAD TO HAVE THE "S" WORD IN IT SOMEHOW.

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DR. SAMBRANO: I DON'T KNOW THAT I CAN FAIRLY ANSWER THAT QUESTION. I WILL TELL YOU THAT JUST, IN GENERAL, KIND OF BEYOND CIRM, IF YOU LOOK AT THE NIH RMAT DESIGNATIONS, WHICH ARE BROADLY REGENERATIVE MEDICINE SOCIETIES AND INSTITUTES WHICH BROADLY DEFINE REGENERATIVE MEDICINE, TYPICALLY INCLUDE GENE THERAPY AS A COMPONENT OF WHAT THEY SUPPORT. SO IT'S PART OF THAT LARGER PACKAGE.

CIRM HAS BEEN UNIQUE AND DIFFERENT. I THINK THE QUESTION IS DOES IT MAKE SENSE FOR CIRM NOW TO EXPAND AS OTHERS HAVE OR AS THE FIELD HAS?

MR. SHEEHY: SO I THINK THIS HAS ALWAYS BEEN A LIVING AGENCY, SO TO SPEAK, AND WE'VE EVOLVED WITH THE SCIENCE. WHEN THIS AGENCY WAS FORMED, I THINK PEOPLE MIGHT HAVE SAID YOU ARE OUT OF YOUR MIND TO TALK ABOUT TAKING A SKIN CELL AND TURNING THAT INTO A PLURIPOTENT CELL. SO INDUCED PLURIPOTENT CELLS EXPLODED ON THE SCENE, AND WE ADAPTED TO WORK WITH THOSE CELLS. AND THE AGENCY -- I THINK AT THE END OF THE DAY THE REAL MISSION IS TO GET CURES.

AND WHEN THIS WAS BROUGHT UP AT THE SCIENCE SUBCOMMITTEE, I WAS A LITTLE AMBIVALENT ABOUT IT. BUT THEN WHEN I STARTED THINKING BACK, WE'VE INVESTED A LOT IN GENE THERAPY. AND GENE

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THERAPY AND REGENERATIVE MEDICINE AND STEM CELLS ALL SEEM TO BE PART OF A BIG POT.

AND IT SEEMED TO ME SOMEWHAT IRRATIONAL TO NOT INCLUDE IN SCOPE SCIENCE THAT WE HAD BROUGHT SO FAR DOWN THE ROAD BECAUSE WE HAVE DONE A LOT OF WORK WITH GENETIC MODIFICATION OF STEM CELLS. IT'S ALL BEEN EX VIVO. AND THEN WHEN WE GET TO THE POINT WHERE WE REALLY HAVE WHAT WOULD BE VERY COMMERCIALLY VIABLE, VERY SCALABLE, WE SAY, OH, WE CAN'T DO THAT. IT JUST DIDN'T FOLLOW FOR ME. SO HAVING THAT OPPORTUNITY AND THE ABILITY TO REALLY GENERATE VERY SCALABLE, WIDELY AVAILABLE, COMMERCIALLY VIABLE CURES THAT WE HAD PLAYED SUCH A ROLE IN DEVELOPING, IT SEEMED TO ME TO BE AN OPPORTUNITY THAT WE WOULD NOT WANT TO MISS.

DR. STEWARD: THANKS. I DID MISS THE SCIENCE SUBCOMMITTEE AND I APOLOGIZE FOR THAT. I WOULD HAVE MADE THESE COMMENTS THERE HAD I BEEN ABLE TO ATTEND.

SO JUST FOLLOWING UP ON YOUR QUESTION, I THINK A COUPLE OF THINGS. THERE ARE A LOT OF THINGS THAT COULD BE CONSIDERED IN THE SAME DOMAIN AS GENE THERAPY IN TERMS OF BEING POTENTIALLY VERY IMPACTFUL FOR TREATMENTS AND CURES FOR ALL KINDS OF DISEASES AND DISORDERS. THAT'S POINT NO. 1.

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SO WHY GENE THERAPY AND NOT OTHER THINGS IS SORT OF A QUESTION. I DON'T REALLY WANT TO GO INTO AN ANSWER TO THAT, BUT I THINK, AS WE ARE CONSIDERING THIS, IT'S IMPORTANT TO TAKE THOSE KINDS OF CONSIDERATIONS. THIS IS A BIG STEP, AND ESPECIALLY A BIG STEP AS WE'RE COMING INTO A POTENTIAL BALLOT INITIATIVE WHERE THINGS ARE GOING TO BE WHATEVER. SO THAT'S SORT OF NO. 2.

AND NO. 3, IN PRINCIPLE, I THINK GENE THERAPY IS GREAT. THE RESEARCH THAT I DO SOMETIMES IS CALLED THAT, SOMETIMES NOT, BUT I THINK THAT TERM HAS VERY BROAD MEANING AND PROBABLY WAY TOO BROAD. AND IF WE WERE TO TAKE THIS STEP, I THINK WE NEED TO BE VERY DEFINITIVE ABOUT WHAT THE DEFINITION IS.

IF IT, FOR EXAMPLE, IS TO TARGET MONOGENETIC DISORDERS THAT AFFECT HUNDREDS OF THOUSANDS OF PEOPLE WORLDWIDE, THAT'S ONE DEFINITION. AND I THINK THAT MAY BE FINE. ALL OF THOSE ARE RARE. THE TERM, IN GENERAL, IS WAY TOO AMBIGUOUS, I THINK, AS IT'S PHRASED RIGHT NOW. THOSE ARE MY THREE COMMENTS. THANK YOU.

MR. SHEEHY: IF I MIGHT RESPOND. THERE IS A CONTROL POINT, AND I KIND OF FEEL LIKE THE CONTROL POINT THAT WE'VE SET UP IS IN LINE WITH PROP 71 AND PERHAPS THE BEST PLACE FOR THE CONTROL POINT TO BE

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ASSIGNED, WHICH IS AT THE GRANTS WORKING GROUP, THE PEER REVIEW GROUP, IN THAT THAT THEY HAVE TO DESIGNATE BY TWO-THIRDS THAT THIS IS A VITAL RESEARCH OPPORTUNITY.

SO I HAVE SOME CONFIDENCE IN THAT. AS YOU KNOW, YOU'VE LISTENED TO AND BEEN PART OF COUNTLESS REVIEWS, THERE IS SOME RELUCTANCE ALWAYS IN THE REVIEW GROUP TO DO THINGS THAT OTHER PEOPLE CAN DO VERY EASILY.

SO I GUESS I FEEL LIKE THAT THAT'S ADEQUATE; BUT, OBVIOUSLY, IF YOU HAVE A DIFFERENT VIEW, SOME WAY TO FIGURE OUT A WAY TO TIGHTEN THE NOOSE, BUT I FEEL THE REVIEW GROUP HAS ALWAYS BEEN PRETTY SKEPTICAL ABOUT DOING SOMETHING THAT ANYBODY ELSE COULD DO EASILY OR COMMERCIALLY.

DR. STEWARD: JUST TO SAY, IF I CAN ADD ONE MORE THING, THAT IS IN FACT SUBSTANTIATED IN PROP 71. IT IS THE STATEMENT THAT STEM CELLS ARE TO BE FUNDED BECAUSE ESSENTIALLY THEY CAN'T BE FUNDED IN ANY OTHER WAY. AND THIS CAN BE FUNDED IN OTHER WAYS. JUST TO SAY, WE'RE CHANGING. I'M NOT SAYING THAT ISN'T A GOOD THING. IT WOULD BE GREAT, IN FACT, TO, IN FACT, REDEFINE REGENERATIVE MEDICINE IN THE NEW BALLOT INITIATIVE, BUT I'M CONCERNED ABOUT DOING IT AT THIS STAGE FOR PROP 71.

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DR. JUELSGAARD: SO LET ME, DR. STEWARD, RESPOND TO THAT BECAUSE ACTUALLY IN THE LANGUAGE OF THE ACTUAL -- GABE MADE A PRESENTATION, BUT THERE'S SOME DEFINITIVE LANGUAGE THAT'S PROVIDED. AND THE LAST PART OF IT, THE LAST GATE TO GET THROUGH, AND THIS IS ACTUALLY GOING TO BE NOW A QUESTION OF GABE OF HOW WE'RE GOING TO DETERMINE THIS, BUT IT ALSO IS BEING DEVELOPED FOR A RARE OR UNMET NEED UNLIKELY TO RECEIVE FUNDING FROM OTHER SOURCES. SO WE'RE KIND OF BACKING INTO THIS NOBODY ELSE IS GOING TO FUND IT.

SO AS TIME HAS GONE ON, THE LIKELIHOOD OF OTHER SOURCES FUNDING STEM CELL RESEARCH HAS CERTAINLY INCREASED WELL AWAY FROM WHERE THINGS STOOD IN 2004, AND WE'VE CONTINUED PROVIDING FUNDING. HOW IS IT THAT WE'RE GOING TO DETERMINE THIS PART OF IT AS UNLIKELY TO RECEIVE FUNDING FROM ANY OTHER SOURCE? WHAT KIND OF DUE DILIGENCE IS GOING TO BE ENGAGED IN TO REQUIRE THAT? BECAUSE I AGREE WE'RE OPENING THE DOOR A BIT POTENTIALLY ON THIS; BUT, AT THE SAME TIME, WE'RE REALLY TRYING TO LIMIT WHAT WE'RE GOING TO LET IN THROUGH THAT SOMEWHAT OPEN DOOR. HOW DO WE MANAGE THAT?

DR. STEWARD: COULD I JUST ADD ONE MORE THING, AND I'M SORRY AND THEN I'LL REALLY BE QUIET,

I PROMISE. ONE COULD SAY THAT ANYTHING THESE DAYS, WITH NIH PAYLINES BEING WHERE THEY ARE, IS UNLIKELY TO BE FUNDED BY NIH. JUST TO SAY.

CHAIRMAN THOMAS: BEFORE WE GET TO OTHER BOARD MEMBERS, DR. SAMBRANO, DO YOU HAVE ANY THOUGHTS ON THAT QUESTION FOR MR. JUELSGAARD?

DR. SAMBRANO: NO. IT'S LANGUAGE THAT'S ALREADY INCLUDED IN OUR ELIGIBILITY REQUIREMENTS FOR OTHER THINGS AS WELL, AND IT WAS FOR THE SMALL MOLECULES AND BIOLOGICS. SO IT'S NOT ALWAYS EASY; BUT, IN GENERAL, THE ARGUMENT HAS BEEN THAT, ESPECIALLY IN THIS AREA OF TRANSLATION AND CLIN1, THERE'S OFTEN LITTLE AVAILABILITY FOR FUNDS TO CARRY THESE PROJECTS FORWARD. SO THAT HAS OFTEN BEEN THE ARGUMENT OR THE RATIONALE BEHIND MOVING SOMETHING THAT IS ADDRESSING AN UNMET NEED IN THIS AREA.

DR. PRIETO: I JUST WOULD COMMENT THAT I THINK THE CALIBER OF THE PEOPLE WHO GIL AND HIS TEAM HAVE RECRUITED FOR THE GRANTS WORKING GROUP INCLUDES PEOPLE WITH A VERY GOOD UNDERSTANDING OF WHAT THE FACTS ON THE GROUND ARE IN ANY PARTICULAR AREA OF THIS RESEARCH. AND THE QUESTION OF STEMNESS OR REGENERATIVENESS OF A PARTICULAR PROPOSAL DOES COME UP AND IS DISCUSSED, I THINK, IN A PRETTY THOUGHTFUL WAY.

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DR. MARTIN: LET ME STATE MY POSITION. I'M CERTAINLY VERY MUCH IN FAVOR OF THIS, BUT THE ISSUE OF UNMET NEED OR DIFFICULT TO SOURCE FUNDING, I THINK, IS AN ISSUE THAT EVOLVES. AND AS WE'VE DISCUSSED JUST IN THE LAST COUPLE OF MINUTES, THE STEM CELL FUNDING INITIALLY WAS NIGH ONTO IMPOSSIBLE IN THIS COUNTRY, AND THAT HAS EVOLVED. AND NOW IT'S COMMONPLACE. AND THERE ARE A LOT OF ACTIVITIES GOING ON, AND I THINK THE WHOLE GENE THERAPY, PARTICULARLY IN VIVO GENE THERAPY, IS NOW DIFFICULT TO GET FUNDED FROM AN AGENCY OR COMMERCIALLY, BUT THAT WILL CHANGE.

AND SO MAYBE THE WAY TO ADDRESS THAT IS TO SAY IT IS DIFFICULT, VERY DIFFICULT ANYWAY, BUT I THINK THAT THE FUNDING OF GENE THERAPY IS JUST IN THE EARLY DAYS, BUT IT WILL BECOME MUCH MORE COMMON AND ACCESSIBLE FOR FUNDING AS IT EVOLVES.

CHAIRMAN THOMAS: OKAY. THANK YOU. ANY OTHER COMMENTS? OKAY. WE NEED A MOTION TO APPROVE.

DR. STEWARD: COULD I JUST MAKE A RECOMMENDATION THAT PERHAPS WE BREAK THIS APART INTO SEPARATE MOTIONS AND MAYBE SEPARATE OUT THIS ONE FROM THE REST? I'D BE A LOT MORE COMFORTABLE.

CHAIRMAN THOMAS: SO WHAT EXACTLY ARE YOU SUGGESTING?

DR. STEWARD: SO I WILL MOVE THAT ALL OF THE CHANGES IN THE CONCEPT PLANS BE APPROVED EXCEPT FOR THE CHANGE WITH REGARD TO THIS WHOLE THING. CAN I MAKE THAT MOTION? IS THAT GETTING COMPLICATED?

MR. TOCHER: NO, I DON'T THINK SO AT THIS POINT. I THINK WE CAN BREAK THAT OUT.

DR. STEWARD: IT MAY VERY WELL BE THAT WE'LL END UP APPROVING BOTH, BUT I'D JUST LIKE TO HAVE THAT AS A SEPARATE VOTE.

CHAIRMAN THOMAS: IS THERE A SECOND?

DR. JUELSGAARD: I'LL SECOND.

CHAIRMAN THOMAS: MOVED BY DR. STEWARD, SECONDED BY MR. JUELSGAARD. IS THERE ANY DISCUSSION, FURTHER DISCUSSION?

DR. MARTIN: WOULD YOU STATE THE MOTION AGAIN? I'M A LITTLE CONFUSED.

DR. STEWARD: EVERYTHING IN THE CONCEPT CHANGE EXCEPT FOR GENE THERAPY.

MR. SHEEHY: WOULD YOU INCLUDE GENE THERAPY OF STEM CELLS? IS THAT STILL IN SCOPE?

DR. STEWARD: OH, ABSOLUTELY. IF IT'S STEM CELL THINGS, SURE. THAT DOESN'T CHANGE ANY OF OUR POLICIES. SO YES.

DR. DULIEGE: BUT THERE WILL BE A SECOND MOTION --

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DR. STEWARD: THERE WILL BE A SECOND.

DR. DULIEGE: -- FOR GENE THERAPY. IT'S NOT THAT WE'RE IGNORING GENE THERAPY, BUT IT'S A SEPARATE MOTION.

MR. TORRES: FOR GUIDANCE, MR. TOCHER, WHAT DOES THE PROPOSITION STATE IN RESPECT TO THIS TYPE OF RESEARCH?

MR. TOCHER: IT WAS ACCURATELY DESCRIBED. WITH RESPECT TO A VITAL RESEARCH OPPORTUNITY, THAT THE AGENCY CAN FUND IF THERE'S A TWO-THIRDS VOTE OF THE GRANTS WORKING GROUP THAT SUCH AN APPLICATION PROVIDES A VITAL RESEARCH OPPORTUNITY.

MR. TORRES: SO WE'RE WITHIN THAT REALM?

MR. TOCHER: ABSOLUTELY.

MS. LANSING: SO WE'RE WITHIN THE REALM, AND I DON'T MIND SPLITTING IT OUT BECAUSE WE'LL VOTE ON IT. I JUST WANT TO SAY THE WHOLE POINT OF THIS AGENCY IS TO MOVE WITH THE SCIENCE AND TO BE AT THE CUTTING EDGE OF THE SCIENCE. AND IF SCIENCE IS MOVING IN ONE WAY AND WE'RE NOT PART OF IT, THEN WE'RE NOT SERVING THE CITIZENS.

CHAIRMAN THOMAS: THANK YOU, SHERRY. OTHER COMMENTS BY MEMBERS OF THE BOARD? ANY PUBLIC COMMENT? HEARING NONE, MARIA, WILL YOU PLEASE CALL THE ROLL.

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MS. BONNEVILLE: GEORGE BLUMENTHAL. LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: YES.

MS. BONNEVILLE: DEBORAH DEAS. DAVID BRENNER. ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING.

MS. LANSING: YES.

MS. BONNEVILLE: LINDA MALKAS.

DR. MALKAS: YES.

MS. BONNEVILLE: BERT LUBIN.

DR. LUBIN: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: YES.

MS. BONNEVILLE: LAUREN MILLER.

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MS. MILLER: YES.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA. FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT. AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: SUZANNE SANDMEYER.
DR. SANDMEYER: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OSWALD STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
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: MOTION CARRIES.
CHAIRMAN THOMAS: THANK YOU. DO WE HEAR A

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SECOND MOTION ON THE GENE THERAPY TOPIC?

MR. SHEEHY: SURE. I WOULD MOVE TO INCLUDE -- MAYBE MR. TOCHER CAN HELP US ON FORM TO INCLUDE THE PIECE WE CUT OUT.

MR. TOCHER: EXCELLENT FORM, JEFF. SURE. THE MOTION IS TO INCLUDE ANY OTHER ENDOGENOUS CELL IF DEEMED A VITAL RESEARCH OPPORTUNITY BY THE CIRM GRANTS WORKING GROUP.

MR. SHEEHY: GENE THERAPY AND ANY OTHER ENDOGENOUS CELL DEEMED A VITAL RESEARCH OPPORTUNITY.

MR. TOCHER: THAT'S RIGHT. THAT LANGUAGE.

MR. SHEEHY: THAT'S MY MOTION.

MR. TORRES: SECOND.

CHAIRMAN THOMAS: SECOND BY SENATOR TORRES. FURTHER DISCUSSION ON THIS TOPIC?

DR. STEWARD: COULD I JUST ASK FOR A FULL EXPLANATION OF WHAT WE MEAN BY GENE THERAPY IN THAT MOTION?

MR. SHEEHY: I THINK I MAY DEFER TO DR. MILLAN TO -- WELL, I'M PERSONALLY -- TO ME THE CONTROL POINT IS THE TWO-THIRDS MAJORITY OF THE GRANTS WORKING GROUP THAT DEEM THIS A VITAL RESEARCH OPPORTUNITY. SO GENE THERAPY OF AN ENDOGENOUS CELL IS A BIG DOOR, BUT WE HAVE A CONTROL POINT TO SHRINK THAT DOOR. SO THAT'S WHY I'M COMFORTABLE WITH A

VERY BROAD DESCRIPTION BECAUSE I REALLY AGREE WITH SHERRY LANSING, THAT WE WANT TO BE ON THE CUTTING EDGE. SO I DON'T WANT TO NARROWLY PRESCRIBE IN ADVANCE WHAT MIGHT COME TO US. AND LET'S NOT FORGET THIS IS VERY HIGH RISK. I MEAN THE GELSINGER CASE WAS AN IN VIVO GENE THERAPY APPROACH. AND SO I THINK THAT IT IS A FRAUGHT FIELD, AND I WOULD LIKE TO BE AT THE CUTTING EDGE BECAUSE I THINK THE PEOPLE WHO ARE GOING TO DO IT ARE PEOPLE WE'VE BEEN FUNDING TO LEARN HOW TO DO IT EX VIVO. AND THE METAPHOR I USED AT THE SCIENCE SUBCOMMITTEE IS I'D HATE TO BRING THE HORSE TO WATER AND THEN SAY YOU CAN'T DRINK AND LET SOMEBODY ELSE GET THAT CREDIT.

DR. STEWARD: LET ME JUST SAY, IF I COULD, IT'S MAYBE NOT BRINGING THE HORSE. IT MAY BE BRINGING A HERD IF WE'RE NOT PRETTY PRESCRIPTIVE IN HOW WE DEFINE THE TERM "GENE THERAPY." AGAIN, NOT TO JUST OVERWHELM THE GRANTS WORKING GROUP, I THINK, YES, THESE ARE EXPERTS, AS YOU KNOW, TO WHOM I ALWAYS GRANT THANKS FOR ALL THEIR HARD WORK. BUT I THINK THAT THIS IS REALLY GOING TO BE A HARD DECISION FOR THEM TO MAKE IN THAT CONTEXT.

ACTUALLY COULD I ALSO JUST ASK ONE QUESTION? HOW MANY PROJECTS OVER THE YEARS HAVE WE FUNDED IN THIS CATEGORY OF -- I FORGET THE EXACT

TERMINOLOGY -- NOT STEM CELLS, BUT THAT REQUIRED A TWO-THIRDS VOTE OF THE SCIENCE SUBCOMMITTEE?

DR. SAMBRANO: WE HAVEN'T.

MR. SHEEHY: I GUESS YOUR PROBLEM THAT YOU IDENTIFY IS A PROBLEM I WOULD LOVE TO HAVE. I WOULD LOVE PEOPLE LINING -- I WOULD LOVE A HERD OF PEOPLE WITH CURES FOR DISEASES INVOLVING IN VIVO GENE MODIFICATION. I THINK THAT THAT WOULD BE A HUGE SUCCESS IF CIRM IN SOME WAY MADE THAT HAPPEN. I MEAN THAT'S WHAT WE ALL HOPE HAPPENS.

SO I WOULD LOVE TO DEAL WITH THAT PROBLEM WHEN IT HAPPENED.

DR. STEWARD: IT'S BETTER THAN A HORSE.

CHAIRMAN THOMAS: DULY NOTED. DR. MARTIN.

DR. MARTIN: THE PROPOSAL, I GATHER, IS NOT OR THE MOTION IS NOT TO RESTRICT THIS TO IN VIVO GENE THERAPY. IT'S GENE THERAPY WHETHER IT BE EX VIVO, IN VIVO, AUTOLOGOUS, OR ALLOGENEIC. IS THAT TRUE?

DR. SAMBRANO: THE COMPONENT THAT REQUIRES THE TWO-THIRDS MAJORITY BY THE GWG IS SPECIFICALLY THE NONSTEM CELLS. SO THIS WOULD BE IN VIVO GENE THERAPY IN AN ENDOGENOUS NONSTEM CELL. OTHER THINGS, SUCH AS WHETHER IT'S EX VIVO OR IN VIVO AND

IT INVOLVES A STEM CELL, WE HAVE BEEN ABLE TO FUND THAT ALREADY. SO IT IS JUST THIS SUBCOMPONENT THAT WE WOULD BE REQUESTING APPROVAL FOR.

DR. MARTIN: BUT WHAT ABOUT EX VIVO GENE THERAPY FOR A NONSTEM CELL, EXACTLY AS YOU HAVE FUNDED FOR STEM CELLS?

DR. SAMBRANO: NO, THAT WOULD NOT BE INCLUDED.

DR. MARTIN: FOR WHAT REASON?

DR. SAMBRANO: THAT THAT WAS NOT THE RECOMMENDATION THAT CAME OUT OF THE SCIENCE SUBCOMMITTEE.

DR. MILLAN: MAY I JUST MAKE A STATEMENT? I THINK A COUPLE OF THINGS THAT HAVE ARISEN IN THE PAST AS WE CONSIDERED PROJECT OPPORTUNITIES THAT CAME TO US THAT WERE NOT ELIGIBLE BECAUSE THIS WASN'T ELIGIBLE IS THAT THERE MAY BE SOME PROMISING APPROACHES TO ADDRESS SIGNIFICANT UNMET MEDICAL NEEDS THAT MAY INVOLVE STEM CELL, BUT MAYBE PREDOMINANTLY AFFECTS THE TARGET POPULATION THAT INDUCES AN EFFICACY AND BENEFIT SIGNAL. AND SO THEN WE COME TO THIS ELIGIBILITY EXERCISE WHERE THEN THE APPLICANTS ARE THEN ASKED TO PROVE THAT IN VIVO IT'S TARGETING THOSE STEM CELLS, AND IT'S A SIGNIFICANT PART OF THE MECHANISM OF ACTION. AND A LOT OF WHAT

HAPPENS AS THESE THERAPIES ARE BEING DEVELOPED AND IT'S BEING MORE ELUCIDATED WHAT THE EFFECT IS IT'S THE CONTRIBUTION OF THE STEM CELL COMPARTMENT, IF THERE IS ONE IN THERE, IS NOT SOMETHING THAT'S REALLY ELUCIDATED UNTIL YOU START DOING CLINICAL TRIALS OFTEN. RIGHT? AND SO YOU'RE KIND OF BETWEEN A ROCK AND A HARD PLACE OF REALLY WHAT HAPPENS IS WE'RE ARTIFICIALLY PUTTING RESTRICTIONS BEFORE WE GET ANSWERS SOMETIMES. THAT'S ONE OF THE THINGS.

ANOTHER OPPORTUNITY HERE, OUR NEXT GENERATION, LET'S SAY WE GET AN HIV CURE, BUT WE WANT TO BE ABLE TO DELIVER IT TO OTHER PARTS OF THE WORLD THAT IS NOT GOING TO BE ABLE TO HANDLE SOME OF THE VERY COMPLEX MANUFACTURING EX VIVO PROCESSES THAT ARE CURRENTLY BEING EXPLORED. IN THE FUTURE, THE NEXT GENERATION MAY BE IN VIVO GENE DELIVERY. AND SO THOSE ARE KIND OF JUST -- I JUST WANTED TO THROW IT OUT THERE IN TERMS OF WHAT THIS COULD ALLOW US TO CONSIDER, AGAIN, PROVIDED THAT IT GOES THROUGH A VERY RIGOROUS PEER REVIEW AND GETS EVALUATED FOR IT OVERCOMES A HURDLE THAT IT IS A VITAL RESEARCH OPPORTUNITY TO ADDRESS AN UNMET MEDICAL NEED THAT'S UNIQUELY ADDRESSED WITH THIS. I JUST WANTED TO PUT THOSE TWO CONSIDERATIONS OUT THERE.

MR. TOCHER: JUST A QUICK CLARIFYING

COMMENT ON OUR PART, NOT IN SUPPORT OR OPPOSITION TO THE MOTION, JUST NOTHING IN THE PROPOSAL CHANGES THE FACT THAT THE APPLICATION REVIEW SUBCOMMITTEE ALWAYS EXERCISES THE FINAL DECISION-MAKING AUTHORITY AS TO WHETHER TO FUND OR NOT FUND ANY AWARD REGARDLESS OF THE GRANTS WORKING GROUP'S RECOMMENDATION.

CHAIRMAN THOMAS: OKAY. IS THERE ANY OTHER DISCUSSION? DR. STEWARD.

DR. STEWARD: I AM SORRY, BUT A QUESTION. SO IF WE HAVE THE ABILITY TO FUND OTHER VITAL RESEARCH OPPORTUNITIES, BUT NEVER HAVE, WHAT WOULD BE THE PROCEDURE BY WHICH THOSE OTHER VITAL RESEARCH OPPORTUNITIES WOULD COME BEFORE CIRM, ONES THAT WERE NOT STEM CELLS OR GENE THERAPY?

MR. SHEEHY: YOU AND ME AND FRANCISCO AND SHERRY HAVE BEEN HERE SINCE THE BEGINNING. WE NEVER PUT IN -- THAT'S ON YOU, OS. ME AND SHERRY AND FRANCISCO, WE NEVER CREATED THAT DOOR. NOW, WE COULD AT SOME POINT CREATE A DOOR, BUT WE HAVEN'T. SO...

DR. STEWARD: YOU COULD IMAGINE THAT THIS DOOR SHOULD BE THERE AND IT ISN'T. AND I'M AGAIN JUST -- I LOVE GENE THERAPY. IT'S GREAT. IT'S INCREDIBLE, BUT I'M JUST CONCERNED THAT, AGAIN, THERE ARE OTHER SHOTS ON GOAL OUT THERE. IF WE'RE

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GOING TO OPEN THE DOOR, LET'S MAKE IT OPEN.

DR. PRIETO: WE HAVE HAD APPLICANTS COME IN IN VARIOUS ROUNDS TRYING TO SLIDE KIND OF SIDEWAYS THROUGH A DOOR THROUGH WHICH THEY REALLY DON'T FIT, MORE THAN ONCE. I THINK THOSE GENERALLY GET WEEDED OUT. SO THERE'S POTENTIALLY AN AVENUE THERE. OH, YEAH. WE'RE REALLY DOING THIS WHATEVER. THAT'S SELF-DECLARED, BUT PERHAPS WE NEED TO CREATE A DOOR.

DR. STEWARD: MY POINT IS THAT THIS WOULD NEVER GET TO THE GRANTS WORKING GROUP. THE EXCLUSION CRITERIA THAT CIRM APPLIES TO THE GRANTS MANAGEMENT PROCESS AT THE FIRST ITERATION WOULD PRECLUDE ANYTHING THAT DOESN'T QUALIFY FROM GETTING THERE. THAT'S MY POINT.

MR. SHEEHY: IN THIS INSTANCE WE ARE CREATING A DOOR.

DR. STEWARD: WE ARE. I KNOW.

MR. SHEEHY: THIS IS THE DECISION POINT WE'RE AT. AGAIN, I JUST THINK LINKING IT TO -- WE'VE BEEN AROUND, WHAT, WHAT ARE WE GOING ON NOW, ALMOST 14, 15 YEARS. WE CREATED SOME CAPACITY. AND WHAT I'M HEARING IS THAT CAPACITY, IN ORDER TO FULLY REALIZE THE PROMISE OF CURES, INCLUDES CREATING THIS DOOR AND THAT'S WHY. THERE IS A LINKAGE TO THE WORK

THAT WE'VE BEEN DOING AS AN AGENCY IN THE PAST. THERE'S A CHAIN OF BEING, SO TO SPEAK, A LINE OF EXPLORATION AND DISCOVERY. THAT'S WHY -- LIKE I SAY, I WOULD HATE TO HAVE PEOPLE WHO HAVE DOING WORK AND GETTING MORE AND MORE SOPHISTICATED TO BE SO SOPHISTICATED THAT THEY WORK THEMSELVES OUT OF ELIGIBILITY AT A POINT WHEN YOU COULD HAVE, AS I SAID, THE MOST SCALABLE, THE MOST COMMERCIALLY VIABLE PROJECTS.

DR. STEWARD: JUST TO BE CLEAR, I'M NOT OBJECTING TO A DOOR. I'M CONCERNED ABOUT THE DEFINITION OF THIS ONE. AND I JUST WONDER IF WE OUGHT TO JUST TAKE A BREATH AND THINK THROUGH THIS A LITTLE BIT MORE CLEARLY. AND THAT IS TELLING YOU HOW I'M GOING TO VOTE ON THIS IF IT COMES TO A VOTE RIGHT NOW. MAKING SURE THAT WE HAVE OUR DEFINITIONS VERY CLEAR, MAKING SURE THAT IF WE DO WANT TO LIMIT IT, THAT'S FINE. MAKE THAT DECISION. IF WE DON'T WANT TO LIMIT IT, THEN THE DOOR IS REALLY THERE FOR OTHER VITAL RESEARCH OPPORTUNITIES THAT COME TO US.

CHAIRMAN THOMAS: DR. SAMBRANO, DO WE HAVE A WORKING DEFINITION?

DR. SAMBRANO: THE ONLY DEFINITION IS WHAT YOU SEE BEFORE YOU OF IN VIVO GENE THERAPY FOR ENDOGENOUS NONSTEM CELLS.

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DR. VUORI: I WAS SORT OF THINKING WHEN LISTENING TO DR. STEWARD'S COMMENTS THAT IF WE THINK ABOUT THE CIRM MISSION, AS THE NAME OF THE ENTITY IMPLIES, I THINK THE GOAL IS TO CARRY OUT REGENERATIVE MEDICINE. AND REALLY WHAT THIS MEANS IS WE ARE TRYING TO REPLACE OR REGENERATE EITHER CELLS, TISSUES, OR ORGANS IN ORDER TO RESTORE OR ESTABLISH THEIR NORMAL FUNCTION. AND SO FAR THE MAIN WAY WE HAVE ADDRESSED THIS IS THROUGH STEM CELLS, RIGHT? AND BASICALLY THIS MAKES SENSE IN STEM CELLS ARE CELLS THAT WHEN PUT BACK INTO THE HUMAN BODY AGAIN DO THAT VERY THING. THEY CAN RESTORE, REGENERATE THE CELL, TISSUE, OR ORGAN FUNCTION THAT WAS NOT THERE.

NOW, THE FIELD OF GENE THERAPY IS MOVING FORWARD VERY RAPIDLY SO THAT IN MY MIND THE VERY DEFINITION OF GENE THERAPY IS THAT NOW WE ARE TRANSFERRING GENETIC MATERIAL INTO CELLS TO PROVIDE THEM WITH THESE NEW FUNCTIONS IN THE BODY. SO IT'S NOT THE STEM CELL THAT GOES IN THERE AND RESTORES THE FUNCTION. THE FIELD OF GENE THERAPY AND IN VIVO GENE THERAPY IS AT A POINT WHERE THE GENETIC MATERIAL TRANSFERRED TO CELLS IS ABLE TO RESTORE, GIVE THEM THIS FUNCTION THAT WAS LOST OR NEVER THERE.

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SO IF IT HELPS, SHOULD WE LIMIT THE DEFINITION OF IN VIVO GENE THERAPY TO MEAN ESSENTIALLY TRANSFER OF GENETIC MATERIAL INTO CELLS IN VIVO WITH THE INTENT TO PROVIDE THEM WITH NEW FUNCTIONS; I.E., REGENERATIVE MEDICINE, SO WE'RE NOT TRYING TO DO EVERYTHING THAT GENE THERAPY CAN BE DOING, BUT REALLY LIVE UP TO THE SPIRIT OF THE REGENERATIVE MEDICINE MISSION OF CIRM.

MR. SHEEHY: SO I WOULD TAKE THAT AS A FRIENDLY AMENDMENT. BUT I WONDER IF WE SHOULD ALSO INCLUDE DR. MARTIN'S, WITH THAT TIGHTER DEFINITION, TO ALSO INCLUDE EX VIVO? YOU CAN IMAGINE CERTAIN CELLS THAT -- A STEP WHERE YOU WOULD TAKE THE CELLS FROM THE BODY TO DO IT BEFORE YOU DO IT IN THE BODY.

DR. MARTIN: I THINK THAT THE LINE IN THE SAND BETWEEN IN VIVO AND EX VIVO IS VERY SOFT. AND I'LL JUST GIVE YOU A SCENARIO. I'M NOT SURE HOW FAR AWAY IT IS. SUPPOSE YOU HAVE A PATIENT WHO IS A GOOD CANDIDATE FOR GENE THERAPY, AND THAT PATIENT HAS, FOR INSTANCE, A TWIN. AND THE TWIN YOU DO EX VIVO GENE THERAPY AND THEN YOU JUST SET UP A SYMBIOSIS BETWEEN THE TWO OF THEM. AND PEOPLE ARE DOING THAT. RIGHT? AND ALL OF A SUDDEN THE THERAPY FOR THE PATIENT WHO'S FUNDED BY CIRM, THAT'S AN IN VIVO THERAPY, BUT IT ACTUALLY WAS EX-VIVO TO BEGIN

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WITH. OR THERE IS A MACHINE, AN INSTRUMENT, THAT'S REMARKABLE THAT MILT TENNY HAS WHERE YOU CAN JUST PUT VIRUS IN THE TOP AND PERIPHERAL BLOOD CELLS IN ANOTHER PORT, AND THAT WILL MAKE CAR-T CELLS AND THEY COME OUT THE BOTTOM.

AND SO YOU HOOK THE PATIENT UP TO JUST THE BLOOD SOURCE, AND NOW IT GOES BACK INTO THE PATIENT. AND THAT'S WORKING. AND IT'S INCREDIBLY EFFICIENT. IT'S SAFER THAN HAVING HUMANS DO THIS MANIPULATION, THE SCIENTIST. SO I THINK THAT THE DIVIDING LINE BETWEEN EX VIVO AND IN VIVO, WHILE YOU MAY BE ABLE TO KNOW IT WHEN YOU SEE IT, NOW I THINK IT'S GOING TO CHANGE. SO I'M NOT SURE THAT WE WANT TO RESTRICT TO JUST EX VIVO EVEN THOUGH THAT WAS THE DECLARATION. AND MAYBE THAT'S SOMETHING WE'LL TAKE ON A YEAR FROM NOW WHEN PEOPLE ARE PUTTING TRANSPOSON I.V. AND THAT'S GOING TO WORK. IT'S CONFUSING RIGHT NOW BECAUSE IT'S EVOLVING.

MR. SHEEHY: SO MAYBE THE MOTION IS TO INCLUDE IN VIVO AND EX VIVO GENETIC MODIFICATION OF ENDOGENOUS CELLS. AND I THINK I'LL HAVE TO RELY ON DR. VUORI FOR THE CLARIFICATION ON WHAT WE EXPECT TO HAPPEN WITH THE GENETIC MODIFICATION. THAT WAS A GREAT ANSWER, BUT I DIDN'T QUITE GET IT ALL LINED OUT, BUT I THOUGHT THAT THAT WAS A VERY CLEAN, NEAT

DEFINITION SO THAT WE'RE NOT SAYING JUST ANYTHING.

DR. MELMED: THERE ARE ENDOGENOUS CELLS, AND THERE COULD BE EXOGENOUS. ENDOGENOUS MEANS NOT SELF.

MR. SHEEHY: SO WE'LL DROP BOTH. WE'LL DROP ENDOGENOUS. DR. VUORI, YOU HAD A DEFINITION. I BELIEVE IT INCLUDED INTRODUCTION OF GENETIC MATERIAL INTO THE CELLS AND AN ELEMENT THAT CHANGED THE FUNCTION OF THE CELLS. THERE MIGHT HAVE BEEN A THIRD TEST, BUT I THINK THOSE ARE AT LEAST TWO OF THE TESTS.

DR. VUORI: UNFORTUNATELY I DIDN'T WRITE ANYTHING DOWN. SOMETHING ALONG THE LINES OF ESSENTIALLY TRANSFER OF GENETIC MATERIAL INTO CELLS IN ORDER TO RESTORE OR ESTABLISH NORMAL CELLULAR FUNCTION OR REGENERATIVE FUNCTION.

DR. MELMED: REGENERATE DISEASED TISSUE.

DR. VUORI: YEAH. SOMETHING ALONG THOSE LINES.

DR. STEWARD: WE CAN'T BE WRITING THESE KINDS OF THINGS ON THE FLY AT A MEETING LIKE THIS, I DON'T THINK. AGAIN, JUST I WOULD RECOMMEND THAT WE SET THIS ASIDE, PUT IT DOWN IN WRITING, GIVE US ALL A CHANCE TO THINK ABOUT IT AND AMEND IT, AND VOTE ON IT. EITHER WE CAN DO IT AT AN AD HOC BOARD MEETING

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OR BY PHONE OR WHATEVER. THERE'S NO RUSH TO DO THIS RIGHT AT THIS MOMENT.

MR. SHEEHY: I ACTUALLY DO THINK THERE'S A RUSH. WE DON'T MEET AGAIN TILL DECEMBER.

DR. STEWARD: DO IT BY PHONE OR SPECIAL PHONE-IN BOARD MEETING.

DR. PRIETO: MR. CHAIRMAN, DO WE HAVE A PROPOSAL IN THE WINGS, SO TO SPEAK, THAT WOULD CREATE AN URGENCY?

MR. SHEEHY: THEY'RE NOT CURRENTLY ELIGIBLE, SO WE DON'T KNOW. I WOULD PREFER TO VOTE -- PERSONALLY -- YOU'RE THE SECOND, I THINK. I WOULD PREFER TO VOTE ON THE MOTION MYSELF.

DR. PRIETO: I'M IN SUPPORT OF THE IDEA, BUT I -- I UNDERSTAND OS' CONCERNS ABOUT TRYING TO WORDSMITH THIS IN THIS KIND OF SETTING. IT'S NOT IDEAL. AND I THINK WE COULD PROBABLY WAIT UNTIL NEXT MONTH. I'D BE CERTAINLY HAPPY TO DO A PHONE MEETING MYSELF.

CHAIRMAN THOMAS: LET ME ASK MR. TOCHER. IS THERE ANY REASON THIS COULD OR COULD NOT BE AN ITEM TO VOTE ON ON THE NEXT TELEPHONIC APPLICATION REVIEW SUBCOMMITTEE?

MR. TOCHER: NO. WE WOULD HAVE TO MAKE SURE THAT WE COULD GET A QUORUM TO ATTEND THAT

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MEETING TELEPHONICALLY, BUT ASSUMING THAT COULD BE DONE.

CHAIRMAN THOMAS: THE MONTHLY CALLS ARE FOR THE FULL BOARD AND THE APPLICATION REVIEW SUBCOMMITTEE.

MS. BONNEVILLE: THE APPLICATION REVIEW SUBCOMMITTEE THAT ATTENDS, WE WOULD JUST NEED TO POLL THE BOARD AND ENSURE THAT WE HAD QUORUM.

CHAIRMAN THOMAS: YES. EVERYBODY HEAR THAT? MR. SHEEHY, IF WE PUT THIS OFF FOR ONE MONTH, WOULD THAT BE OKAY WITH YOU?

MR. SHEEHY: SURE.

CHAIRMAN THOMAS: OKAY. THANK YOU.

DR. STEWARD, SO THE IDEA -- MR. SHEEHY HAS SAID HE'S OKAY WITH THE IDEA OF PUTTING THIS OFF FOR A MONTH TILL THE NEXT TELEPHONIC MEETING OF THE BOARD AND THE APPLICATION REVIEW SUBCOMMITTEE WITH AN UNDERSTANDING THAT WE NEED TO GET ENOUGH PEOPLE ON THAT CALL WHO AREN'T ON THE APPLICATION REVIEW SUBCOMMITTEE SO THAT WE HAVE A QUORUM OF THE BOARD.

MS. BONNEVILLE: THAT'S ON NOVEMBER 15TH AT ELEVEN IN THE MORNING JUST SO EVERYONE WRITES THAT DOWN.

CHAIRMAN THOMAS: OKAY. MR. SHEEHY.

MR. SHEEHY: MIGHT I ASK IF DR. MILLAN AND

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CIRM TEAM COULD FACILITATE COMING UP WITH A DEFINITION BASED ON SOME OF THE CONVERSATIONS WE'VE HAD HERE TODAY?

DR. MILLAN: YES, WE WILL.

DR. SANDMEYER: ALONG THOSE LINES, WOULD IT BE POSSIBLE TO HAVE SEVERAL EXAMPLES OF WHAT WOULD NOT QUALIFY FOR IN VIVO GENE THERAPY UNDER THIS NEW INVITATION?

MR. SHEEHY: I WITHDRAW THE MOTION.

CHAIRMAN THOMAS: MOTION IS WITHDRAWN. THE SECOND WITHDRAWN AS WELL. IT'S BEEN A VERY GOOD, ROBUST DISCUSSION. THANK YOU, DR. STEWARD, FOR YOUR THOUGHTS. AND THANK YOU, MR. SHEEHY, FOR YOUR LEADERSHIP ON THIS IDEA. THANK YOU, DR. VUORI, FOR YOUR ARTICULATION. WE WILL HAVE THIS AS A CALENDARED ITEM FOR THE NOVEMBER APPLICATION REVIEW AND BOARD CALL.

SO ON TO ITEM NO. 11.

MS. BONNEVILLE: TEN AND 11 WERE TOGETHER.

MS. WINOKUR: MAY I ASK A QUESTION? WHO WILL BE WORKING ON THIS IN THE MEANTIME?

DR. MILLAN: DIANE, THE CIRM TEAM WILL CIRCULATE PROPOSED LANGUAGE TO THE BOARD.

MS. WINOKUR: THANK YOU.

DR. MILLAN: YOU'RE WELCOME.

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CHAIRMAN THOMAS: OKAY. I BELIEVE WE'RE GOING TO SKIP TO NO. 15, CORRECT?

MS. BONNEVILLE: LET'S DO ITEM NO. 9 A.M., HAVE EVERYONE GRAB LUNCH, AND THEN COME BACK.

CHAIRMAN THOMAS: FAIR ENOUGH. ITEM NO. 9 A.M., CONSIDERATION OF APPOINTMENT OF CO-CHAIRS TO THE EVALUATION SUBCOMMITTEE. THIS IS FOR ME TO DISCUSS.

SO IN THE ORDINARY COURSE, WE CONDUCT AN ANNUAL REVIEW OF OUR CEO'S PERFORMANCE AND WANTED TO ENGAGE THE EVALUATION SUBCOMMITTEE TO DO THAT. AND TOWARDS THAT END, I HAVE ASKED DR. GASSON AND MR. JUELSGAARD IF THEY WOULD BE CO-CHAIRS FOR THAT EVALUATION SUBCOMMITTEE GOING FORWARD. SO THE SOLE PURPOSE OF THIS MOTION, WHICH WE NEED ONE, IS TO APPOINT THEM IN THOSE CAPACITIES.

THE EVALUATION SUBCOMMITTEE IS UNUSUAL AMONGST SUBCOMMITTEES AS REQUIRING A BOARD VOTE FOR THIS SORT OF THING IN CASE ANYBODY IS WONDERING WHY THIS AN AGENDIZED TOPIC.

SO I WOULD MOVE THAT WE SO APPOINT DR. GASSON AND MR. JUELSGAARD AS CO-CHAIRS OF THE EVALUATION SUBCOMMITTEE. IS THERE A SECOND?

DR. LUBIN: SECOND.

CHAIRMAN THOMAS: SECONDED BY DR. LUBIN.

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ANY DISCUSSION? ANY PUBLIC COMMENT? HEARING NONE, IS THIS SOMETHING THAT REQUIRES A ROLL CALL OR IS VOICE VOTE SUFFICIENT, MR. TOCHER?

MR. TOCHER: WE SHOULD DO A ROLL CALL VOTE AND WITH THOSE ON THE PHONE.

CHAIRMAN THOMAS: ROLL CALL EVEN IN THE ROOM.

MS. BONNEVILLE: GEORGE BLUMENTHAL. LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: YES.

MS. BONNEVILLE: DEBORAH DEAS. DAVID BRENNER. ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS. SHERRY LANSING.

MS. LANSING: YES.

MS. BONNEVILLE: LINDA MALKAS.

DR. MALKAS: YES.

MS. BONNEVILLE: BERT LUBIN.

DR. LUBIN: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: SHLOMO MELMED.

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DR. MELMED: YES.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA. FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT. AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: SUZANNE SANDMEYER.
DR. SANDMEYER: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OSWALD STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: KRISTINA VUORI.
DR. VUORI: YES.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.

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MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN THOMAS: OKAY. WE ACTUALLY ARE GOING TO ADJOURN FOR LUNCH IN TWO SECONDS, BUT WE HAVE A FIVE-SECOND ITEM, WHICH IS NO. 13. LET'S JUST GET THROUGH THAT AS WELL. APPOINTMENT OF SCIENTIFIC MEMBERS TO THE GWG.

AS ALWAYS ON A MONTHLY BASIS, WE HAVE NEWLY IDENTIFIED POTENTIAL MEMBERS OF THAT. IT'S NORMALLY A CONSENT ITEM. IT'S LISTED HERE AS AN ACTION ITEM. SO DO I HAVE A MOTION TO APPROVE?

MS. LANSING: I MOVE IT.

DR. MALKAS: SECOND.

CHAIRMAN THOMAS: SECONDED BY DR. MALKAS. IS THERE ANY DISCUSSION ON THIS? ANYBODY WANT DR. SAMBRANO TO NAME WHO IT IS, OR WE JUST TRUST THAT, AS UNUSUAL, HE'S COME UP WITH AUGUST ADDITIONS TO OUR GROUP?

DR. SAMBRANO: YOU SHOULD HAVE THE BRIEF BIO OF DR. ALAN ROBBINS IN YOUR MATERIALS.

CHAIRMAN THOMAS: ANY DISCUSSION? ANY PUBLIC COMMENT? ALL THOSE IN THE ROOM IN FAVOR PLEASE SAY AYE. OPPOSED? ABSTENTIONS? MARIA, WILL YOU CALL THE ROLL OF THOSE ON THE PHONE.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: YES.

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MS. BONNEVILLE: DAVID HIGGINS. SHERRY LANSING.

MS. LANSING: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: YES.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: THANK YOU. MOTION CARRIES.

CHAIRMAN THOMAS: OKAY. THANK YOU. SO WE'RE GOING TO CONVENE NOW. WE WOULD LIKE MEMBERS TO GET THEIR LUNCH AND BRING IT BACK INTO THE ROOM. WHEN WE DO RESUME, WE'RE GOING TO GO INTO ITEMS INVOLVING THE APPLICATION REVIEW SUBCOMMITTEE STARTING WITH ITEM 15 AND THEN PROCEEDING TO ITEMS 5 AND 6, WHICH WILL BE CHAIRED BY MR. SHEEHY. SO IF EVERYBODY COULD GET THEIR LUNCH AND PLEASE COME BACK AND WE WILL RESUME SHORTLY. THANK YOU.

(A RECESS WAS TAKEN.)

CHAIRMAN THOMAS: OKAY. WE'RE GOING TO RESUME. GOING TO START, AS I MENTIONED, WITH ITEM

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15, WHICH IS IN REFERENCE TO THE DISCOVERY AWARDS. BEFORE WE TURN IT OVER TO MR. SHEEHY, MR. TOCHER HAS A COMMENT.

MR. TOCHER: THAT'S RIGHT. SO AT THE MOMENT NOW THE BOARD IS SITTING AS THE APPLICATION REVIEW SUBCOMMITTEE. THIS PARTICULAR ITEM, ITEM 15, BECAUSE IT CAME TO THE BOARD'S ATTENTION AFTER THE ORIGINAL NOTICE WAS POSTED TEN DAYS AGO, WE JUST HAVE A FORMALITY WE NEED TO ENGAGE IN IN ORDER TO CONSIDER THESE QUEST ITEMS. SO WE JUST NEED TO TAKE A VOTE ON TWO COMPONENTS: ONE, THAT THE APPLICATION REVIEW SUBCOMMITTEE NEEDS TO TAKE IMMEDIATE ACTION TO ENSURE NO UNDUE DELAY IN THE PROGRESSION OF THIS RESEARCH AND, SECOND, THAT THE NEED FOR THIS ACTION, CONSIDERATION OF THESE QUEST AWARDS, CAME TO OUR ATTENTION AFTER THE ORIGINAL AGENDA WAS POSTED.

CHAIRMAN THOMAS: DO YOU NEED --

MR. TOCHER: MEMBERS OF THE APPLICATION REVIEW SUBCOMMITTEE TO MAKE, SECOND, AND VOTE ON THAT MOTION.

CHAIRMAN THOMAS: SO GIVE OVER TO MR. SHEEHY.

MR. SHEEHY: SO COULD I GET A MOTION TO THAT EFFECT FROM ONE OF THE MEMBERS OF THE APPLICATION REVIEW SUBCOMMITTEE?

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DR. DULIEGE: MOVE IT.

DR. MARTIN: SECOND.

MR. SHEEHY: A MOTION FROM DR. DULIEGE AND A SECOND FROM DR. MARTIN. DO WE NEED A VOICE VOTE, SCOTT?

MR. TOCHER: THAT'S RIGHT AND A ROLL FOR THOSE ON THE PHONE.

MR. SHEEHY: ALL THOSE IN FAVOR. AND THEN FOR THE PHONE.

MS. BONNEVILLE: SHERRY LANSING. LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: THANK YOU.

MR. SHEEHY: SO THAT MOTION PASSES.

NOW WE'RE MOVING INTO ITEM 15?

CHAIRMAN THOMAS: MR. TOCHER, DO WE NEED TWO MOTIONS?

MR. TOCHER: NO. THAT ONE WAS FINE. YOU CAN CONSIDER THE ITEM NOW.

MR. SHEEHY: DR. SAMBRANO HAS A PRESENTATION.

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DR. SAMBRANO: WE ARE NOW GOING TO CONSIDER THE CIRM QUEST DISCOVERY PROGRAM APPLICATIONS. AND I'M GOING TO GIVE YOU JUST A BRIEF REMINDER OF WHAT THE QUEST PROGRAM IS ABOUT, WHICH IS KIND OF OUR ENGINE OF DISCOVERY. IT TAKES PROMISING NEW STEM CELL-BASED TECHNOLOGIES THAT WILL BE READY FOR TRANSLATIONAL WORK WITHIN ABOUT TWO YEARS. THE IDEA IS TO GET THEM TO DEVELOP A CANDIDATE THAT CAN BE ADVANCED TO TRANSLATION AS QUICKLY AS POSSIBLE.

NOW, SOME OF THESE APPLICATIONS HAVE GONE THROUGH A LONG JOURNEY TO GET HERE, AND SO I'M JUST GOING TO DESCRIBE A LITTLE BIT OF THAT HISTORY.

SO THE GRANTS WORKING GROUP REVIEWED THESE APPLICATIONS AND ORIGINALLY RECOMMENDED 14 OF THEM FOR FUNDING. SO THE TOTAL APPLICANT REQUEST WAS ABOUT 19 MILLION; HOWEVER, THE FUNDS THAT WERE AVAILABLE WERE ONLY TEN.

SO WHAT HAPPENED AT A JULY 19 ICOC APPLICATION REVIEW SUBCOMMITTEE WAS THAT $10 MILLION OF FUNDING WAS APPROVED FOR EIGHT APPLICATIONS, AND THAT INCLUDED PARTIAL FUNDING FOR TWO OF THEM. AND THEN THE DECISION ON THE REMAINING SIX WAS DEFERRED. AND THEN SUBSEQUENT TO THAT MEETING, CIRM RECOVERED SOME FUNDS FROM ONE OF THE PARTIALLY FUNDED

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APPLICATIONS THAT DIDN'T REALLY GO THROUGH. THE PROJECT PI MOVED OUT OF STATE, AND SO THE PARTIAL FUNDS WERE RECOVERED AND BROUGHT BACK INTO THE POOL OF FUNDS.

AND THEN LAST WEEK ON OCTOBER 18TH THE APPLICATION REVIEW SUBCOMMITTEE CONSIDERED THESE APPLICATIONS AGAIN, APPROVED FUNDING OF 549,000 OR 550 OR SO TO COMPLETE THE TOTAL AMOUNT REQUESTED FOR ONE OF THE PARTIALLY FUNDED APPLICATIONS, WHICH WAS DISC2-18109, AND THEN, BECAUSE THAT ESSENTIALLY UTILIZED ALMOST ALL THE FUNDS, DEFERRED DECISION ON THE REMAINING SIX APPLICATIONS. SO THOSE SIX HAVE BEEN CARRIED FORWARD AND STILL COULD NOT ACT UPON IT, BUT DID SO BY RECOMMENDING USE OF FUNDS FROM THE 2019 BUDGET TO FUND THOSE SIX APPLICATIONS. SO THAT'S WHY YOU CONSIDERED THAT EARLIER TODAY. CLEARLY THAT WAS APPROVED. AND SO WE NOW HAVE A BUDGET THAT WILL ALLOW FUNDING OF THOSE SIX REMAINING APPLICATIONS.

AND SO OUR RECOMMENDATION IS TO MOVE FORWARD WITH FUNDING THOSE APPLICATIONS, AND THE TOTAL THAT WOULD BE UTILIZED FOR THOSE WOULD BE 7.9 MILLION FOR THOSE APPLICATIONS. MR. SHEEHY.

MR. SHEEHY: THANK YOU, DR. SAMBRANO.

SO DO WE HAVE A MOTION FROM THE

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APPLICATION REVIEW SUBCOMMITTEE TO ACCEPT THE TEAM'S RECOMMENDATION AND FUND THOSE REMAINING APPLICATIONS?

DR. JUELSGAARD: SO MOVED.

MR. SHEEHY: DO I HAVE A SECOND?

DR. MARTIN: SECOND.

MR. SHEEHY: MR. JUELSGAARD HAS MADE THE MOTION; DR. MARTIN HAS SECONDED IT. DO WE HAVE DISCUSSION? PUBLIC COMMENT?

DR. STEWARD: BEFORE YOU ACTUALLY ASK FOR PUBLIC COMMENTS, THIS IS A QUESTION. DO WE WANT TO -- ARE WE MOVING THEM ALL?

MR. SHEEHY: YES.

DR. STEWARD: CAN I JUST HAVE THE MAKER OF THE MOTION TO COMMENT ON THAT IN SUPPORT OF THAT MOTION?

MR. JUELSGAARD: THAT'S UP TO THE CHAIR. I HAVE NO POWER TO CONTROL WHAT YOU SAY OR DON'T SAY. PLEASE, OS, GO AHEAD.

MR. SHEEHY: DID YOU HAVE A QUESTION?

DR. STEWARD: NO. THE QUESTION WAS THE JUSTIFICATION FOR MAKING THE MOTION FOR ALL OF THESE RATHER THAN MAYBE CONSIDERING THEM ONE AT A TIME. THAT'S MY QUESTION.

MR. JUELSGAARD: JUSTIFICATION WAS THAT

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THEY WERE ALL TIER I PROJECTS WHEN THEY WERE PRESENTED TO US SOME TIME AGO. AND WE ADDRESSED THEM ONE AT A TIME LAST TIME BECAUSE WE DIDN'T HAVE ENOUGH MONEY TO DO THEM ALL, AND WE RECOGNIZED THAT AB INITIO. SO WE SAID, OKAY, WELL, LET'S TAKE THEM ONE BY ONE BY ONE.

IF YOU WANT TO --

(INTERRUPTION BY PHONE OPERATOR.)

MR. JUELSGAARD: I'LL ADMIT IT WAS ON MY SIDE, THAT INTERNAL ERROR. I'LL TAKE THE FALL FOR IT, OS.

SO IF YOU WANT TO AMEND THE MOTION TO EXCLUDE CERTAIN APPLICATIONS FROM THE MOTION, IT'S SOMETHING YOU CAN RAISE.

DR. STEWARD: NO. JUST TO SAY THAT WOULD BE KIND OF THE NORMAL WAY WE DO THIS. IS THERE A MOTION TO NOT FUND ONE? JUST MY QUESTION.

MR. SHEEHY: CAN I ANSWER IT?

DR. STEWARD: YEAH.

MR. SHEEHY: BECAUSE WE DID HAVE AN APPLICATION REVIEW SUBCOMMITTEE IN THE INTERIM WHERE WE HAD THIS DISCUSSION. AND THAT'S WHERE THERE WAS KIND OF A DEBATE WHETHER TO ACTUALLY ASK FOR ADDITIONAL MONEY. WE ACTUALLY MET TO REALLOCATE -- TO DO TWO THINGS: TO REALLOCATE THE RETURNED FUNDS

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TO THE PROJECT THAT WE ONLY HALF FUNDED. AND THEN THE DISCUSSION THERE WAS DO WE -- DO WE TAKE A POSITION ON THESE REMAINING APPLICATIONS. WHAT CAME FROM THERE WAS TO FUND THE REST OF THE APPLICATIONS. SO THAT'S KIND OF WHAT'S DRIVING THIS MEETING IS THAT THAT DISCUSSION DID NOT START PICKING THEM OUT AND SAYING WE WANT TO FUND. THE REQUEST THEN CAME. THE RECOMMENDATION WAS TO ASK THE FULL BOARD FOR THE FULL FUNDS FOR ALL THE APPLICATIONS. SO THAT'S THE GENESIS OF THAT.

DR. STEWARD: THAT WAS AT THE SCIENCE SUBCOMMITTEE THOUGH, RIGHT?

MR. SHEEHY: NO. IT WAS THE APPLICATION REVIEW SUBCOMMITTEE.

DR. STEWARD: NO. WHAT WE DID THERE WAS ACTUALLY SAY THAT WE WILL CARRY THEM FORWARD. WE DIDN'T ACTUALLY VOTE TO FUND THEM, I BELIEVE. I'D HAVE TO GO BACK.

MR. SHEEHY: I KNOW. THE MEETINGS WERE ON THE SAME DAY. I CAN'T REMEMBER WHAT HAPPENED.

MR. TOCHER: THE MOTION WAS TO CONTINUE CONSIDERATION UNTIL THEY COULD BE FUNDED FROM BRIDGE FUNDS THAT MIGHT BE RAISED. SO THAT WAS THE MOTION THAT WAS APPROVED AT THE APPLICATION REVIEW SUBCOMMITTEE.

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DR. STEWARD: SO THAT WAS MY RECOLLECTION. THAT'S WHY I'M ASKING. JUST AGAIN.

MR. SHEEHY: SO WE CAN REVIEW THESE INDIVIDUALLY. WE DO HAVE A MOTION TO CONSIDER THEM ALL, BUT THAT'S UP TO THE MAKER OF THE MOTION AND THE SECOND WHETHER THEY WANT TO WITHDRAW THAT AND WE CONSIDER THESE INDIVIDUALLY.

DR. JUELSGAARD: BEFORE I DECIDE, CAN I GET A LITTLE MORE FEEDBACK AS TO -- BECAUSE IF WE DO THESE ONE BY ONE, IT'S GOING TO TAKE UP A FAIR AMOUNT MORE TIME POTENTIALLY.

DR. STEWARD: I'M NOT ACTUALLY SUGGESTING THAT. I'M JUST REALLY SAYING THAT NORMALLY WHAT WE DO IS TO GO THROUGH THAT PROCESS OF ARE THERE ANY OF THIS GROUP THAT WE DO NOT WANT TO FUND.

DR. JUELSGAARD: WHAT WE NORMALLY DO IS I THINK WE SAY MOVE THEM TO TIER II OR SOMETHING LIKE THAT.

DR. STEWARD: WHATEVER THE LANGUAGE IS. AND THAT MAY BE --

DR. JUELSGAARD: WE DID THAT AT THE MEETING. WE NEVER MOVED ANY OF THESE BACK TO TIER I AT THE MEETING IN WHICH WE ONLY PARTIALLY FUNDED THEM.

DR. STEWARD: BUT THAT WAS BECAUSE WE

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CONTINUED CONSIDERATION OF THEM.

MR. JUELSGAARD: I'M GOING TO LET MY MOTION REST AS IS. I'M NOT GOING TO WITHDRAW IT, WHICH IS TO APPROVE AT THIS POINT ALL SIX.

DR. MARTIN: I'LL APPROVE TOO.

MR. SHEEHY: SO WE HAVE THAT MOTION. SINCE THAT'S ON THE FLOOR, IS THERE ANY FURTHER DISCUSSION OF THE MOTION BY BOARD MEMBERS? IS THERE ANY PUBLIC COMMENT?

DR. REED: I KNOW LOGICALLY THAT CIRM CANNOT WORK ACTUAL MAGIC. YOU CANNOT MAKE MONEY COME WHEN THERE ISN'T SOME. BUT LAST WEEK IT FELT PRETTY CLOSE TO THAT. WHEN THE BOARD SAID ZERO MONEY, AND THEN ALL OF A SUDDEN A WAY WAS FOUND TO WHERE THESE FIVE GENUINELY OUTSTANDING PROJECTS ARE ALL GOING TO BE FUNDED, THAT'S GOING TO STICK IN MY MIND FOREVER. I'M NOT CONNECTED TO ANY OF THEM. I READ THEM ALL. ONE ON LIVER WAS GREAT; THE ONE ON BLADDER CANCER WAS TERRIFIC. THIS IS IMPORTANT STUFF AND YOU FOUND A WAY. ON BEHALF OF PATIENT ADVOCATES THANK YOU ALL.

MR. KHALID: GOOD AFTERNOON, GUYS. MY NAME IS ALI. I'M HERE FOR DISC2-18070 FOR AUTISM TREATMENT BEHALF OF DR. LIPTON STUART. AND I WAS HERE LAST WEEK AS WELL AND I EXPLAINED MYSELF. AND

I REALLY APPRECIATED THAT YOU GUYS ARE GIVING ME A CHANCE TO SAY SOMETHING ONE MORE TIME. THIS IS A LIFE-CHANGING MOMENT FOR ME, FOR MY FAMILY, AND LOT OF PEOPLE OUT THERE LIKE THIS WHO KIDS ARE GOING THROUGH THIS DAILY BASIS BECAUSE WE CAN UNDERSTAND WHAT WE GO THROUGH EVERY DAY AND WHAT WE'RE DEALING WITH.

SO THIS IS A HUGE THING FOR US. AND WE DON'T HAVE NO OTHER HOPE BESIDE WHAT DR. LIPTON STUART DOING FOR MY SON AND FOR OTHER KIDS RELATED TO THAT DISEASE, MEF2C. SO THAT'S THE ONLY THING I WANT TO ADD. I HAVE NOTHING ELSE TO SAY. THIS IS UP TO YOU GUYS. THAT'S ALL. THANK YOU.

MR. SHEEHY: THANK YOU. THANK YOU FOR YOUR COMMENTS.

ANY OTHER PUBLIC COMMENT? SEEING NONE, CAN WE CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: ABSTAIN.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
MR. SHEEHY: GREAT. THANK YOU.
I BELIEVE NOW WE GO TO ITEM NO. 5 ON THE AGENDA, WHICH IS CONSIDERATION OF THE CLINICAL TRIAL STAGE PROJECTS. AND I THINK -- WILL DR. PATEL BE TAKING US THROUGH THOSE?
DR. PATEL: YES.
MR. SHEEHY: THANK YOU. OR THROUGH THAT ONE. I THINK WE HAVE ONE SINGLE PROJECT THERE.

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DR. PATEL: SO MY NAME IS SHYAM PATEL, AND THANK YOU VERY MUCH FOR GIVING ME THE OPPORTUNITY TO PRESENT THE CLINICAL PROGRAM TO YOU TODAY. IT'S BEEN A LITTLE WHILE SINCE WE BROUGHT CLIN APPLICATIONS TO YOU, AND WE'RE VERY EXCITED TO BRING THIS ONE TO YOU TODAY.

SO, AS YOU KNOW, THE CLINICAL PROGRAM IS ACTUALLY COMPOSED OF THREE DIFFERENT OPPORTUNITIES. THERE'S IND-ENABLING CLIN1 OPPORTUNITY, CLIN2 IS FOR PHASE 1, 2, AND 3 CLINICAL TRIAL PROJECTS, AND CLIN3 IS FOR REGISTRATION ACTIVITIES ON AN ONGOING CLIN2 AWARD. TODAY WE'RE PRESENTING A CLIN1 APPLICATION TO YOU FOR APPROVAL.

JUST A REMINDER, THE SCORING MECHANISM THAT OUR GRANTS WORKING GROUP USES FOR THE CLIN PROGRAM, THIS IS A THREE-TIER SCORING SYSTEM AS OPPOSED TO OUR OTHER FUNDING MECHANISMS. A SCORE OF 1 WOULD INDICATE THE APPLICATION HAS EXCEPTIONAL MERIT AND WARRANTS FUNDING; A SCORE OF 2 INDICATES IT HAS SOME MINOR FLAWS THAT REQUIRE ADDRESSING PRIOR TO IT BEING AWARDED, AND A SCORE OF 3 INDICATES IT HAS MAJOR FLAWS AND SHOULD NOT BE FUNDED AT THIS TIME AND SHOULD NOT BE RESUBMITTED FOR SIX MONTHS.

TO GIVE YOU AN IDEA OF THE CLINICAL

BUDGET, WE STARTED THIS YEAR WITH A $130 MILLION IN THE ANNUAL BUDGET FOR THE CLIN PROGRAM. TO DATE YOU APPROVED $86 MILLION IN APPROVED AWARDS FOR THE CLIN PROGRAM. TODAY'S APPLICATION FOR YOUR CONSIDERATION IS A $4 MILLION AWARD ROUGHLY. IF YOU WERE TO APPROVE THAT, IT WOULD LEAVE $40 MILLION FOR THE REST OF THE YEAR.

SO WHEN WE STARTED THIS YEAR, CIRM TEAM SET INITIAL INTERNAL TARGETS FOR CLIN 2 AND CLIN1 AWARDS. FOR CLIN2 THAT WAS TWELVE. WE'RE AT SIX SO FAR. CLIN1 IT WAS FOUR. WE'VE ALREADY MET THAT TARGET, AND THIS IS GOING TO BE THE FIFTH CLIN1 AWARD.

SO THE ACTUAL APPLICATION UP FOR CONSIDERATION TODAY IS CLIN1-18223. THIS IS A LATE STAGE PRECLINICAL STUDIES PROJECT FOR A THERAPY FOR HIV/AIDS. THE THERAPY ITSELF IS GENETICALLY ENGINEERED CAR-T CELLS. THESE ARE CMV-SPECIFIC HIV TARGETING CAR-T CELLS. INDICATION, AGAIN, IS FOR HIV/AIDS. AND THE GOAL FOR THIS PROJECT IS TO OPTIMIZE THE MANUFACTURING, TO CONDUCT PRECLINICAL SAFETY AND EFFICACY STUDIES, NEEDS TO BE IND-ENABLING STUDIES, AND TO PREPARE AND SUBMIT THE IND ITSELF. THE FUNDS REQUESTED ARE $3.8 MILLION WITH ZERO DOLLARS IN COFUNDING. JUST A REMINDER,

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THE MAXIMUM FUNDS ALLOWABLE FOR THIS CATEGORY ARE $6 MILLION.

SO TO GIVE YOU AN IDEA ABOUT THIS PROJECT AND GIVE YOU SOME BACKGROUND ON IT, THE POTENTIAL IMPACT IS THAT THERE ARE 1.1 MILLION PEOPLE IN THE U.S. LIVING WITH HIV, AS MANY OF YOU ALREADY KNOW. THERE ARE APPROXIMATELY 40,000 NEWLY DIAGNOSED PATIENTS EACH YEAR. AND THIS IS ACCORDING TO THE NIH. AND APPROXIMATELY 16,000 HIV PATIENTS DIE IN THE U.S. EACH YEAR. COULD BE FOR VARIOUS REASONS.

SO THE VALUE PROPOSITION FOR THIS PARTICULAR THERAPY IS THE FOLLOWING: THE CURRENT STANDARD OF CARE, AS YOU ALL KNOW, IS ANTI-RETROVIRAL THERAPY. AND WHILE ART IS EFFECTIVE AT CONTROLLING HIV, IT REQUIRES DAILY ADMINISTRATION OF THE DRUGS, AND IT'S ASSOCIATED WITH VARIOUS MORBIDITIES, INCLUDING CARDIOVASCULAR DISEASE AND CANCER. SO THE PROPOSED CAR-T THERAPY WOULD BE POTENTIALLY A SINGLE ADMINISTRATION OF THE CAR-T CELLS THAT COULD ACHIEVE COMPLETE, WHICH IS A STERILE CURE, OR A FUNCTIONAL CURE OF HIV INFECTION WITHOUT THE NEED FOR ART. SO THEY WOULD NOT NEED THE DAILY ADMINISTRATION OF THIS DRUG POTENTIALLY.

THE STEM CELL RELEVANCE IS THAT THE CELL THERAPY ITSELF IS COMPOSED OF CENTRAL MEMORY AND

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MEMORY STEM T CELLS.

WE DO HAVE A COUPLE OF OTHER PROJECTS IN OUR PORTFOLIO RIGHT NOW THAT ARE ACTIVE IN THE CLINICAL STAGE THAT ARE ALSO TARGETING THE SAME INDICATION. THERE ARE BOTH PHASE 1 AND PHASE 1/2 TRIALS IN HIV/AIDS OR AIDS LYMPHOMA, AND BOTH OF THESE ARE USING GENE-MODIFIED HEMATOPOETIC STEM CELLS MEANT TO BE RESISTANT TO HIV INFECTION.

AND, LASTLY, THIS PARTICULAR APPLICATION HAS NOT RECEIVED PREVIOUS FUNDING FROM CIRM FOR EARLIER STAGES OF THE PROJECT.

THE GWG REVIEWED THIS APPLICATION, AND THEY FOUND IT TO HAVE EXCEPTIONAL MERIT AND IT WARRANTED FUNDING. AND THEY UNANIMOUSLY SCORED IT A TIER I SCORE WITH 12 VOTES. THERE WERE NO VOTES FOR TIER II AND NO VOTES FOR TIER III. AND THE CIRM TEAM CONCURS WITH THE GWG RECOMMENDATION FOR THE FUNDING AMOUNT OF $3,812,797.

MR. SHEEHY: THANK YOU. SO DO I HAVE A MOTION TO ACCEPT THE GWG AND THE CIRM TEAM RECOMMENDATION?

MR. TORRES: MOVE IT.

MR. SHEEHY: SENATOR TORRES. DO I HAVE A SECOND?

CHAIRMAN THOMAS: SECOND.

MR. SHEEHY: SECONDED BY CHAIRMAN THOMAS.
DO WE HAVE ANY BOARD DISCUSSION? ANY PUBLIC COMMENT? CAN WE CALL THE ROLL.
MS. BONNEVILLE: ANNE-MARIE DULIEGE.
DR. DULIEGE: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO. AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.

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CAN YOU HOLD THIS OPEN?

MR. SHEEHY: SURE. I WILL SAY AN INTERESTING THING ABOUT THIS THAT DIDN'T GET HIGHLIGHTED IS THE CMV REACTIVITY, WHICH WILL ACTUALLY PLAY A ROLE, COULD ACTUALLY -- IT'S ONE OF THE MORE INTERESTING THINGS BECAUSE YOU DON'T NEED TO DO CONDITIONING, THAT YOU'RE ABLE TO PROLIFERATE AND MAINTAIN THE COMPARTMENT BECAUSE OF THE CMV REACTIVITY. AND THAT ACTUALLY COULD HAVE APPLICATION TO ALL CAR-T CELL THERAPIES, GETTING RID OF THE NEED TO USE CONDITIONING IN ORDER TO USE THOSE CELLS. SO I THOUGHT THAT WAS A REALLY COOL ASPECT OF THIS IS THAT THERE WAS A FEATURE TO IT THAT WAS VERY INNOVATIVE.

DR. PRIETO: ARE YOU WAITING FOR MY VOTE?

MR. SHEEHY: WE'RE WAITING FOR YOUR VOTE.

DR. PRIETO: AYE.

MR. SHEEHY: WE HAVE AN AYE FOR DR. PRIETO.

MS. BONNEVILLE: DIANE WINOKUR.

MR. SHEEHY: IT PASSES. GREAT. THANK YOU.

SO COULD WE GO NOW TO ITEM NO. 6, THE TRANSLATION PRESENTATION, AND I THINK IS THAT DR. SAMBRANO?

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DR. SAMBRANO: YEAH. IT'S ME.

MR. SHEEHY: WE DID PUBLIC COMMENT.

DR. SAMBRANO: THANK YOU, MR. SHEEHY. SO THIS IS THE TRANSLATION PROGRAM. JUST A REMINDER OF WHERE IT FITS AMONG OUR FUNDING OPPORTUNITIES. TRANSLATION IS RIGHT AT THE CENTER, TAKING PROJECTS THAT COME OUT OF DISCOVERY THAT HAVE A CANDIDATE AND TAKES THEM TO THE POINT WHERE THEY'RE READY TO CONDUCT IND-ENABLING STUDIES. SO THAT IS THE OBJECTIVE. OF COURSE, WE WANT TO SUPPORT PROMISING STEM CELL-BASED PROJECTS THAT WILL ACCELERATE THE COMPLETION OF THESE TRANSLATIONAL ACTIVITIES AND ADVANCE THEM AS QUICKLY AS WE CAN TOWARDS THE CLINIC.

AS I HAD MENTIONED PREVIOUSLY, THE TRAN PROGRAM SUPPORTS CANDIDATES THAT COVER A VARIETY OF PRODUCT TYPES, INCLUDING THERAPEUTICS DEVICES, DIAGNOSTIC, MEDICAL DEVICES, AND TOOLS. IN THIS PARTICULAR CASE, AS IT USUALLY IS THE CASE, THEY ARE PRIMARILY THERAPEUTIC. WE HAD ONE APPLICATION THAT WAS FOR A TOOL. SO FOR THE MOST PART, WE'RE GOING TO DISCUSS THERAPEUTIC APPLICATIONS.

SO IN THAT CONTEXT, THE TRANSLATION PROGRAM WILL TAKE SOMETHING THAT HAS IDENTIFIED A SINGLE CANDIDATE THAT SHOWS DISEASE-MODIFYING

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ACTIVITIES AS THE READINESS POINT FOR COMING INTO THE PROGRAM. WHEN IT'S FUNDED, IT IS ALLOWED ABOUT 30 MONTHS TO GET TO THE EXPECTED OUTCOME OF COMPLETING A PRE-IND MEETING WITH THE FDA.

THE REVIEW CRITERIA THAT ARE UTILIZED TO ASSESS THESE BY THE GWG INCLUDE THE OVERALL SIGNIFICANCE AND POTENTIAL FOR IMPACT OF THE PROJECT; THAT IS, WHAT VALUE IT BRINGS; THE RATIONALE, WHETHER THAT IS SOUND, MAKES SENSE, AND HAS SUPPORTING DATA TO CONTINUE MOVING THIS FORWARD; WHETHER IT'S WELL-PLANNED AND DESIGNED; AND, OF COURSE, WHETHER IT'S FEASIBLE, INCLUDING HAVING A QUALITY TEAM AND ALL THE RESOURCES AVAILABLE TO CARRY THE PROJECT OUT.

THE SCORING SYSTEM, UNLIKE THE CLINICAL PROGRAM THAT DR. PATEL PRESENTED, GOES BACK TO THE ONE TO A HUNDRED SCORING SYSTEM. SO HERE APPLICATIONS THAT ARE DEEMED MERITORIOUS HAVE A SCORE OF 85 OR ABOVE. THOSE THAT ARE NOT RECOMMENDED FOR FUNDING HAVE A MEDIAN SCORE BETWEEN 1 AND 84.

SO THE RECOMMENDATIONS THAT CAME OUT OF THIS CYCLE OF TRAN FROM THE GRANTS WORKING GROUP IS THAT THERE ARE THREE APPLICATIONS THAT WERE RECOMMENDED FOR FUNDING. THE TOTAL APPLICANT

REQUEST FOR THOSE THREE APPLICATIONS IS ABOUT 13.5 MILLION, AND WE HAVE ABOUT 15.8 AVAILABLE TO COVER THESE AMOUNTS REQUESTED. SO THERE ARE SUFFICIENT FUNDS TO COVER THOSE THREE.

AND SO, BRIEFLY, IF YOU WANT ME TO GO THROUGH EACH ONE, OR WE CAN SHOW THE SPREADSHEET THAT SHOWS THOSE THREE THAT ARE RECOMMENDED.

MR. SHEEHY: WHAT ABOUT THIS AS A WAY TO PROCEED? MAYBE I'LL TAKE A MOTION TO SEE ABOUT MOVING ANY FROM TIER II INTO TIER I. AND THEN WE CAN TAKE THEM EACH ONE BY ONE AND HAVE A VOTE ON EACH APPLICATION SINCE THERE'S ONLY THREE OF THEM.

SO IS THERE ANYONE WHO WISHES TO MOVE AN APPLICATION FROM TIER II TO TIER I? SINCE THERE'S NO MOTION TO THAT EFFECT, DR. SAMBRANO, CAN WE START WITH THE FIRST RECOMMENDED APPLICATION.

DR. SAMBRANO: FIRST APPLICATION THAT'S RECOMMENDED IS TRAN-18265. SO THIS ONE IS ENTITLED "CLINICAL TRANSLATION OF AUTOLOGOUS REGENERATIVE CELL THERAPY FOR BLINDNESS." IT IS ONE OF TWO VISION-RELATED APPLICATIONS THAT ARE BEING RECOMMENDED.

THIS ONE IS ADDRESSING BROADLY MACULOPATHY, SO THOSE THINGS THAT INCLUDE AGE-RELATED MACULAR DEGENERATION, MYOPIC MACULAR

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DEGENERATION, AND STARGARDT'S DISEASE. AND THE THERAPY APPROACH IS AN AUTOLOGOUS INDUCED PLURIPOTENT STEM CELL-DERIVED RETINAL PIGMENT EPITHELIUM THAT WOULD BE TRANSPLANTED IN ORDER TO TREAT PATIENTS WITH THESE MACULOPATHIES.

SO THE SCORE THAT WAS GIVEN BY THE GWG WAS AN 85. OUT OF THE 14 MEMBERS THAT SCORED THIS APPLICATION, 13 SCORED WITHIN THE FUNDING RANGE. THERE WAS JUST ONE THAT SCORED BELOW THAT.

SO OVERALL THE GRANTS WORKING GROUP WAS ENTHUSIASTIC ABOUT THIS APPLICATION. THERE WERE SOME MINOR COMMENTS RELATED TO THE OVERALL RATIONALE IN TERMS OF PRELIMINARY DATA AND POSSIBLE CHALLENGE TO COMMERCIALIZATION, BUT OTHERWISE A STRONG RECOMMENDATION FOR THIS APPLICATION.

MR. SHEEHY: DO I HAVE A MOTION TO ACCEPT THE GRANTS WORKING GROUP'S RECOMMENDATION ON THIS APPLICATION?

DR. DULIEGE: MOVE.

MR. ROWLETT: SECOND.

MR. SHEEHY: DO WE HAVE ANY DISCUSSION ON THAT APPLICATION AMONG BOARD MEMBERS? IS THERE ANY PUBLIC COMMENT ON THAT APPLICATION? CAN WE CALL THE ROLL PLEASE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: THANK YOU. THE MOTION CARRIES.

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MR. SHEEHY: THANK YOU. NEXT APPLICATION PLEASE.

DR. SAMBRANO: NEXT APPLICATION IS TRAN1-11259, SO THIS ONE IS ENTITLED "DEVELOPING ENGINEERED AUTOLOGOUS LEUKEMIA VACCINES TO TARGET RESIDUAL LEUKEMIC STEM CELLS." SO WHAT THIS PROJECT DOES IS IT TAKES PATIENT-SPECIFIC LEUKEMIA CELLS FOR PATIENTS WITH AML. SO THIS IS AN APPLICATION THAT TAKES FROM AML PATIENTS LEUKEMIA CELLS AND CREATES A VACCINE FROM THEM. SO THEY ENGINEER THESE CELLS TO EXPRESS SOME NOVEL IMMUNE STIMULATORY MOLECULES WITHIN THOSE CELLS AND INTRODUCE THEM BACK TO THE PATIENT. THEY ARE, OF COURSE, RADIATED BEFORE BRINGING THEM BACK TO THE PATIENT. AND THE IDEA IS THAT THEY STIMULATE AND DEVELOP AN IMMUNE RESPONSE TO THEIR AML. AGAIN, AN AUTOLOGOUS APPROACH TO THIS.

THIS APPLICATION RECEIVED A SCORE OF 85. WE HAD TEN MEMBERS OF THE GWG THAT SCORED BETWEEN 85 AND A HUNDRED, AND THERE WERE THREE THAT SCORED IT BETWEEN 1 AND 84.

(PAUSE IN PROCEEDINGS.)

DR. SAMBRANO: LET ME USE THIS MICROPHONE WHILE THAT IS RESOLVED.

SO I THINK WHERE I LEFT OFF IS WE HAD TEN

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MEMBERS OF THE GWG THAT SCORED THIS APPLICATION BETWEEN 85 AND 100 AND THREE THAT GAVE IT A SCORE BETWEEN 1 AND 84. SO OVERALL ANOTHER STRONG APPLICATION FROM THE VIEW OF THE GRANTS WORKING GROUP. THERE WERE SOME MINOR CONCERNS ABOUT THE EXTENT TO WHICH THIS MIGHT BE SPECIFICALLY TARGETING OR BE A STEM CELL-TARGETING PRODUCT. AND SOME REVIEWERS ASKED FOR ADDITIONAL PRELIMINARY DATA IN TERMS OF SUPPORTING THE EFFICACY, BUT OTHER THAN THAT, A STRONG RECOMMENDATION FOR THIS.

MR. SHEEHY: SO DO WE HAVE A MOTION TO ACCEPT THE GWG'S RECOMMENDATION?

MR. TORRES: MOVE IT.

DR. DULIEGE: SECOND.

MR. SHEEHY: ANY DISCUSSION ON THIS? SO WE HAVE A MOTION. WE HAD A SECOND, DR. DULIEGE. DO WE HAVE ANY BOARD DISCUSSION ON THIS? ANY PUBLIC COMMENT? CAN WE CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER.

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MS. MILLER: YES.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO. AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES. OH, FRANCISCO.
DR. PRIETO: AYE.
MS. BONNEVILLE: THANK YOU.
MR. SHEEHY: DR. SAMBRANO, COULD WE GO TO THE NEXT APPLICATION.
DR. SAMBRANO: ABSOLUTELY. SO THE NEXT APPLICATION IS TRAN1-18300. SO THIS ONE IS ENTITLED "A PURIFIED ALLOGENEIC CELL THERAPY PRODUCT FOR

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TREATMENT OF DRY AGE-RELATED MACULAR DEGENERATION."
SO THIS IS THE SECOND OF THE TWO VISION-RELATED
APPLICATIONS THAT WERE RECOMMENDED.

THIS ONE IS INTENDED TO DEVELOP AN
ALLOGENEIC PRODUCT AS OPPOSED TO AN AUTOLOGOUS ONE
FOR RETINAL PIGMENT EPITHELIAL CELLS IN ORDER TO
RESTORE FUNCTION TO THE RETINA SPECIFICALLY FOR DRY
AGE-RELATED MACULAR DEGENERATION.

SO THIS APPLICATION WAS RECOMMENDED WITH A
SCORE OF 85 WITH 12 MEMBERS OF THE GWG GIVING IT A
SCORE BETWEEN 85 AND 100, THREE THAT SCORED BELOW
THAT. MINOR CONCERNS ONLY REALLY WITH THIS
APPLICATION REQUESTING ADDITIONAL DATA ON
PRELIMINARY DATA THAT MIGHT HELP DETERMINE WHETHER
THE SINGLE CELLS, BECAUSE THEY'RE DEVELOPING A
SINGLE CELL PRODUCT AS OPPOSED TO ONE THAT COMES IN
THE FORM OF NEUROSPHERES, WOULD BE ACTUALLY BETTER.
SO FEELING THAT A LITTLE MORE DATA WOULD BE NEEDED
FOR THESE. OTHER THAN THAT, A STRONG RECOMMENDATION
FROM THE GWG.

MR. SHEEHY: THANK YOU, DR. SAMBRANO. DO
WE HAVE A MOTION TO ACCEPT THE GWG RECOMMENDATION?

DR. PRIETO: SO MOVED.

MR. TORRES: SECOND.

MR. SHEEHY: IS THERE ANY DISCUSSION

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AMONGST BOARD MEMBERS?

DR. MARTIN: JUST HAVE A TECHNICAL QUESTION. IN A SITUATION WHERE IT'S AGE RELATED, WHY IS AN ALLOGENEIC CELL THERAPY GOING TO BE SUPERIOR TO AUTOLOGOUS CELLS?

DR. SAMBRANO: IT'S A GOOD QUESTION. I DON'T KNOW THAT I CAN ANSWER THAT.

DR. MARTIN: BECAUSE IT'S PROBABLY NOT GENETICALLY DETERMINED. EVEN IF IT IS, YOU NEED A COUPLE OF YEARS.

DR. JUELSGAARD: COST.

MR. SHEEHY: ADDITIONAL DISCUSSION? PUBLIC COMMENT? CAN WE CALL THE ROLL PLEASE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: LAUREN.
MOTION CARRIES.
MR. SHEEHY: THE MOTION CARRIES. AND I BELIEVE THAT CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW SUBCOMMITTEE.
MR. TOCHER: ACTUALLY, JEFF, WE HAVE JUST ONE LAST MOTION TO CLOSE OUT THE APPLICATIONS IN TIER II. IF WE COULD JUST HAVE A MOTION FROM SOMEONE WHO ISN'T OS STEWARD TO NOT FUND ALL REMAINING APPLICATIONS, THAT WILL BE MADE AND SECONDED BY SOMEONE ELSE. AND, OS, YOU CAN PARTICIPATE EXCEPT FOR THOSE WITH WHICH YOU HAVE A CONFLICT.
MR. SHEEHY: GREAT. COULD I GET SOMEONE

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TO MAKE THE MOTION TO CLOSE.

MR. TORRES: MOVE IT.

DR. PRIETO: SECOND.

MR. SHEEHY: ANY BOARD COMMENT? ANY PUBLIC COMMENT? CAN WE CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

MR. 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.

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MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
MR. SHEEHY: GREAT. NOW THAT CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW SUBCOMMITTEE.
CHAIRMAN THOMAS: THANK YOU, MR. SHEEHY.
LAST ITEM ON THE AGENDA IS -- LISA, ARE YOU IN HERE? COULD SOMEBODY SEE IF WE COULD GET -- HERE SHE COMES. WE'RE GOING TO HAVE A CLINICAL PROGRAM UPDATE BY DR. KADYK.
DR. KADYK: THANK YOU, DR. THOMAS AND MEMBERS OF THE BOARD AND MEMBERS OF THE PUBLIC, AND FELLOW TEAM MEMBERS. I HAVE THE HONOR TODAY TO REPRESENT THE THERAPEUTICS TEAM TO GIVE THE CLINICAL UPDATE. TODAY WE'RE GOING SO BE FOCUSING ON THE CIRM HEMOGLOBINOPATHY PROGRAMS, WHICH IS AN AREA THAT'S ACTUALLY GROWN QUITE A BIT. JUST IN THE PAST YEAR, WE'VE HAD FIVE NEW AWARDS IN VARIOUS STAGES FROM TRANSLATION UP THROUGH CLINICAL TRIALS IN THIS AREA. AND IT'S ACTUALLY A GOOD EXAMPLE OF HOW SOME OF THESE THERAPIES THAT WE'RE FUNDING COULD POTENTIALLY BE CURATIVE AND ALSO IMPACT DISEASES BEYOND THE DISEASES THAT ARE BEING INVESTIGATED

HERE.

SO BASICALLY THREE MESSAGES THAT I WANT TO GET ACROSS TODAY. THE FIRST IS THAT THERE'S A HUGE UNMET NEED FOR TREATING THESE HEMOGLOBINOPATHIES. SECOND, CIRM IS FUNDING A NUMBER OF DIFFERENT APPROACHES TO TREATING THESE, ANY ONE OF WHICH WOULD NOT ONLY TREAT, BUT POTENTIALLY CURE THESE DISEASES. SO THEY'RE OF HUGE IMPACT. AND, THIRD, IF THEY'RE SUCCESSFUL, THEY COULD EASILY BE ADAPTED TO TREATING A HUGE NUMBER OF OTHER GENETIC DISEASES OF THE BLOOD. SO IT'S A REALLY, I THINK, IMPORTANT SET OF AWARDS THAT WE'RE FUNDING HERE.

SUFFICE IT TO SAY I THINK THAT THIS PROGRAM IS A GREAT EXAMPLE OF HOW CIRM IS ACCELERATING STEM CELL TREATMENTS OR EVEN CURES TO PATIENTS WITH UNMET MEDICAL NEEDS.

BEFORE I GET INTO HEMOGLOBINOPATHIES, I JUST WANT TO GIVE YOU A BRIEF OVERVIEW OF THE WHOLE THERAPEUTIC PORTFOLIO AS IT STANDS TODAY. SO YOU CAN SEE OUR CANONICAL PIE CHART WITH ALL THE VARIOUS DIFFERENT DISEASE AREAS THAT WE'RE FUNDING. WE FUNDED 49 CLINICAL TRIALS TO DATE SINCE THE INCEPTION OF CIRM. THIS PIE CHART ALSO INCLUDES 12 AWARDS THAT ARE AT THE SO-CALLED CLIN1 OR IND-ENABLING STAGE. SO IND IS THE INVESTIGATIONAL

NEW DRUG APPLICATION THAT'S REQUIRED TO BE FILED WITH THE FDA IN ORDER TO DO A CLINICAL TRIAL.

AND SO TODAY I'M GOING TO BE TALKING ABOUT THE GREEN SLICE OF THE PIE WHICH IS WHERE THE HEMOGLOBINOPATHIES FIT IN THE BLOOD DISEASES.

SO WANT TO FIRST GIVE A LITTLE BACKGROUND FOR THOSE OF YOU WHO MAY NOT BE AS FAMILIAR WITH THESE DISEASES. THEY'RE A FAMILY OF SEVERE OR FATAL DISEASES THAT ARE ALL CAUSED BY DEFECTS IN THE HEMOGLOBIN MOLECULE. AND THE HEMOGLOBIN MOLECULE IS IN THE RED BLOOD CELLS, AND IT'S THE MOLECULE THAT'S RESPONSIBLE FOR CARRYING OXYGEN TO ALL THE TISSUES OF YOUR BODY.

SO THE THREE DIFFERENT DISEASES THAT ARE IN THIS FAMILY THAT I'M GOING TO TALK ABOUT TODAY ARE SICKLE CELL DISEASE, WHICH, OF COURSE, WAS DISCUSSED EARLIER TODAY AND I'LL GO INTO A LITTLE MORE DETAIL ABOUT THE PROGRAMS THAT WE'RE ALREADY FUNDING THERE. IT'S CAUSED BY A DEFECTIVE FORM OF THAT BETA HEMOGLOBIN MOLECULE.

BETA THALASSEMIA IS CAUSED BY TOO LITTLE OF THE BETA HEMOGLOBIN SUBUNIT, AND ALPHA THALASSEMIA IS CAUSED BY TOO LITTLE OF THE ALPHA HEMOGLOBIN SUBUNIT.

SO SICKLE CELL DISEASE. IT'S A HUGE UNMET

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NEED. IT AFFECTS AROUND 100,000 PEOPLE IN THE U.S. AND INCLUDING ABOUT 7,000 PEOPLE IN CALIFORNIA. IT'S MUCH MORE HIGHLY PREVALENT IN AFRICA, AND THERE'S VERY HIGHER RATES IN AFRICAN-AMERICANS AND HISPANICS. SO AS I MENTIONED, IT'S A DEFECT IN THE BETA GLOBIN GENE. AND YOU CAN SEE ON MY DIAGRAM, IT'S CALLED THE SICKLE FORM OF THE BETA HEMOGLOBIN GENE BECAUSE IT CAUSES THE NORMALLY ROUND RED BLOOD CELLS TO FORM THIS SICKLE SHAPE, CAUSES THE RED BLOOD CELLS TO DIE EARLY, WHICH CAUSES ANEMIA, BUT ALSO THAT SHAPE TENDS TO CLOG THE SMALLER BLOOD VESSELS, WHICH BASICALLY CUTS OFF THE OXYGEN SUPPLY TO TISSUES AND ORGANS. AND IN ADDITION TO THE SEVERE ANEMIA, THERE'S ALSO SEVERE PAIN CRISES THAT OCCUR AND STROKE AND ORGAN DAMAGE THAT CAN RESULT FROM THIS DISEASE.

THERE ARE SOME TREATMENTS THAT ARE OUT THERE NOW THAT PATIENTS CAN USE, BUT THEY'RE NOT CURATIVE. AND SO THERE IS A SHORTENED AVERAGE LIFE SPAN FOR PATIENTS IN THE U.S., AROUND 40 YEARS ON AVERAGE. THAT SAID, THAT'S WAY LONGER THAN IN AFRICA WHERE TYPICALLY CHILDREN DIE WHEN THEY'RE FIVE OR SIX YEARS OLD.

SO HIGHLIGHT TODAY A LITTLE MORE DETAIL ABOUT FOUR OF OUR SICKLE CELL PROGRAMS. TWO ARE

PHASE 1/2, TWO ARE IN THE PRECLINICAL STAGES. SO DR. KOHN AT UCLA, DR. ROSENTHAL AT CITY OF HOPE, DR. PORTEUS AT STANFORD, AND DR. WALTERS AT CHILDREN'S HOSPITAL.

BEFORE WE TALK ABOUT THAT, BETA THALASSEMIA IS ALSO CAUSED BY A DEFECT IN THAT BETA SUBUNIT OF HEMOGLOBIN. IT ALSO CAUSES SEVERE LIFELONG ANEMIA, REQUIRING FREQUENT BLOOD TRANSFUSIONS WHICH IN AND OF THEMSELVES CAN CAUSE MORBIDITIES. AND, AGAIN, PATIENTS WITH THIS DISEASE HAVE A SHORTENED LIFE SPAN. AND WE HAVE RECENTLY LAUNCHED A CLINICAL TRIAL SPONSORED BY ED CONNER AND SANGAMO TO TRY AND TREAT BETA THALASSEMIA.

AND, FINALLY, ALPHA THALASSEMIA IS THE VERSION IN WHICH THE ALPHA SUBUNITS ARE DEFECTIVE. THIS IS A LITTLE BIT MORE OF A RARE DISEASE. IT'S PARTLY, I THINK, BECAUSE OF MANY OF THE CASES ARE MISSED BECAUSE FETUSES DIE IN UTERO BEFORE THEY'RE EVEN BORN OF HEART FAILURE OR MAY BE TERMINATED BECAUSE OF POOR PROGNOSIS. IN ORDER FOR AN ALPHA THALASSEMIA PATIENT TO SURVIVE, THEY HAVE TO, AGAIN, HAVE THESE REGULAR RED BLOOD CELL TRANSFUSIONS WHICH CAUSE COMORBIDITIES.

SO I'LL TALK TO YOU ALSO ABOUT DR. TIPPI MACKENZIE'S TRIAL AT UCSF TO ADDRESS THIS

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THALASSEMIA. SO I CATEGORIZED THE VARIOUS CIRM-FUNDED APPROACHES INTO THREE MAJOR CATEGORIES. I FORGOT TO MENTION ONE CRITICAL THING, WHICH IS THAT ALL OF THESE DISEASES CAN BE CURED TODAY, BUT THE CURE IS A BONE MARROW TRANSPLANT OR A HEMATOPOIETIC STEM CELL TRANSPLANT. AND THE PROBLEM IS, FIRST OF ALL, IT'S VERY HARD TO FIND AN APPROPRIATE DONOR FOR THESE PATIENTS. YOU WANT TO HAVE AN IMMUNE MATCH. AND WITHOUT A GOOD MATCH, TYPICALLY THERE ISN'T A GOOD MATCH. EVEN IF THERE IS, THE CONDITIONING REGIMEN FOR A BONE MARROW TRANSPLANT IS VERY TOXIC IN AND OF ITSELF WITH A RISK OF DEATH. AND MANY OF THESE PATIENTS ARE TOO SICK TO EVEN UNDERGO THE PROCEDURE.

SO ALL OF THESE APPROACHES THAT I'M GOING TO TELL YOU ABOUT TODAY ARE WAYS TO MAKE A KINDER, GENTLER BONE MARROW TRANSPLANT SO THAT MANY MORE PATIENTS WOULD BE ABLE TO UNDERGO THE TRANSPLANT AND HAVE IT BE CURATIVE.

SO WE HAVE TWO AWARDS WHICH I BEND INTO THE CATEGORY OF HAVING A HALF-MATCHED OR RELATED DONOR TRANSPLANT. ONE IN WHICH THERE'S MORE TRADITIONAL GENE THERAPY, WHICH IS GENE ADDITION TO THE PATIENT'S OWN BLOOD STEM CELLS. AND THEN, THREE, EARLIER STAGE AWARDS IN WHICH THEY'RE LOOKING

AT GENE EDITING OF PATIENT'S OWN BLOOD STEM CELLS TO CURE THE DISEASES.

SO FOR THE HALF-MATCHED TRANSPLANT, WE'VE GOT AN AWARD TO JOSEPH ROSENTHAL AT CITY OF HOPE. HE'S GOT A PHASE 1 TRIAL THAT STARTED RECENTLY. AND THE IDEA HERE IS THAT BY DOING A TRANSPLANT WITH A DONOR SUCH AS A SIBLING OR A PARENT WHICH HAS PARTIAL MATCH, IT'S BEEN SHOWN THAT YOU CAN GET COEXISTENCE OF BOTH THE DONOR AND RECIPIENT CELLS WITHOUT HAVING GRAFT VERSUS HOST DISEASE OR OTHER SORTS OF MORBIDITIES. THEY CAN CO-EXIST. THE DONOR CELLS CAN CURE THE DISEASE. AND THE BEAUTY IS THAT THERE'S A MILDER CONDITIONING METHOD USED TO GENERATE THESE SO-CALLED MIXED CHIMERAS SO THAT OLDER PATIENTS WHO'VE ALREADY GOT MORE SEVERE DISEASE COULD POTENTIALLY BE TREATED. SO HIS TRIAL IS UNDER WAY, AND THEY'RE LOOKING AT SAFETY AS WELL AS POTENTIAL EFFICACY.

THE SECOND TRIAL I'M GOING TO TELL YOU ABOUT IS A VARIATION ON THE THEME. IT'S ALSO A HALF-MATCHED BLOOD CELL TRANSPLANT, BUT IT'S DONE IN UTERO. THIS IS BY TIPPI MACKENZIE AT UCSF. AND HERE SHE IS USING MATERNAL BLOOD CELLS TO TRANSPLANT INTO THE FETUS BEFORE IT'S BORN IN ORDER TO CURE THE DISEASE. AND THE BEAUTY HERE IS THAT YOU DON'T NEED

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TO CONDITION THE FETUS. THE IMMUNE SYSTEM IS STILL BEING DEVELOPED, SO IT'S NOT LIKELY TO REJECT THE MATERNAL CELLS. IF THERE'S SUFFICIENT ENGRAPHMENT OF THE MATERNAL CELLS, IT MAY CURE THE DISEASE. AND FAILING THAT, IT STILL MAY BE POSSIBLE AFTER BIRTH TO DO A TRANSPLANT FROM THE MOTHER WHICH WOULD REQUIRE MUCH MILDER CONDITIONING BECAUSE THE FETUS WAS ALREADY EXPOSED TO THE MATERNAL ANTIGENS IN UTERO.

AGAIN, THIS IS A PHASE 1/2 LOOKING PRIMARILY AT SAFETY, BUT ALSO THE FEASIBILITY AND EFFICACY OF THIS TREATMENT.

THE NEXT CATEGORY IS THE GENE ADDITION TO PATIENT'S OWN STEM CELLS. YOU'RE PRETTY FAMILIAR WITH THIS APPROACH FROM DON KOHN. OF COURSE, EVIE UP THERE ON THE WALL WAS CURED BY DON KOHN OF ADA SKID USING A VERY SIMILAR APPROACH OF TAKING OUT THE PATIENT'S BLOOD STEM CELLS, ADDING IN A NORMAL COPY OF THE GENE, AND THEN REPLACING THE STEM CELLS TO CURE THE DISEASE. SO WORKING ON THAT FOR SICKLE CELL.

AND, FINALLY, THERE'S A FEW GROUPS THAT ARE WORKING ON GENE EDITING OF PATIENT'S STEM CELLS. THIS IS SIMILAR TO THE GENE THERAPY APPROACH THAT I JUST DESCRIBED EXCEPT IT'S USING SPECIAL ENZYMES TO

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ACTUALLY EDIT THE PATIENT'S DNA TO CORRECT IT. SO THE APPROACH THAT SANGAMO THERAPEUTICS USES IS ZINC FINGER NUCLEASES TO DO THE GENE EDITING. AND IN THEIR CASE THEY'RE FINDING A WAY TO INDUCE A DIFFERENT FORM OF HEMOGLOBIN THAT CAN REPLACE THE ONE THAT'S MISSING, THE FETAL HEMOGLOBIN. SO THAT'S A SAFETY STUDY THAT'S JUST INITIATED.

MATT PORTEUS AT STANFORD IS FINISHING UP A CLIN1 AWARD, SO IND-ENABLING SAFETY STUDIES AND MANUFACTURING FOR A CLINICAL TRIAL, AND IS PLANNING TO FILE AN IND APPLICATION SOON. HE IS USING, INSTEAD OF ZINC FINGERS, HE'S USING THE NEW CRISPR-CAS9 GENE EDITING METHOD TO CORRECT THE ACTUAL DEFECTIVE GENE IN THE PATIENT'S BLOOD STEM CELLS.

AND SIMILARLY, MARK WALTERS AT CHILDREN'S HOSPITAL OAKLAND IS ALSO USING CRISPR-CAS9 TO CORRECT THE PATIENT'S OWN BLOOD STEM CELLS. HE'S AT THE TRANSLATIONAL STAGE AND IS GETTING READY TO HOLD A PRE-IND MEETING WITH THE FDA.

SO I MENTIONED TIPPI MACKENSIE. SHE'S DOING THESE IN-UTERO TREATMENTS. SO HOW DO YOU KNOW THAT THE BABY HAS THIS DISEASE BEFORE YOU DO THE TREATMENT? SO THIS IS A COMPLEMENTARY AWARD. I THINK THAT'S REALLY APPROPRIATE FOR THIS PORTFOLIO

IS AN AWARD TO CASSANDRA CALLOWAY AT CHILDREN'S HOSPITAL WHO IS DEVELOPING A NEW, NONINVASIVE WAY OF DIAGNOSING THESE HEMOGLOBINOPATHIES.

TYPICALLY NOW IT REQUIRES AMNIOCENTESIS OR CHORIONIC VILLI SAMPLING WHICH PUTS THE FETUS AT RISK. AND THIS METHOD SHE'S DEVELOPING WOULD BE BASICALLY TAKING A BLOOD SAMPLE FROM THE MOTHER AND SEQUENCING THE FETAL DNA THAT IS CIRCULATING WITHIN THE MOTHER -- IT'S PRETTY AMAZING -- TO SCREEN FOR MUTATIONS. AND THIS TYPE OF A DIAGNOSTIC WOULD BE INVALUABLE FOR AN APPROACH SUCH AS DR. MACKENSIE'S AT UCSF.

SO THAT'S OUR CURRENT PORTFOLIO. I WANT TO REITERATE THE MAIN THREE TAKE-HOME MESSAGES HERE, WHICH IS, FIRST OF ALL, THESE HEMOGLOBINOPATHIES ARE A MAJOR UNMET NEED. THERE ARE MULTIPLE APPROACHES TO A CURE THAT ARE BEING INVESTIGATED WITH CIRM FUNDING. AND ANY OF THESE APPROACHES COULD LEAD TO SAFER TREATMENTS AND MAKE THEM MORE AVAILABLE TO MANY, MANY MORE PATIENTS.

AND, FINALLY, SUCCESS WITH THESE METHODS COULD TRANSLATE TO OTHER GENETIC BLOOD DISEASES AND REALLY HAVE A MUCH BROADER IMPACT THAN JUST THE HEMOGLOBINOPATHIES.

AND WITH THAT, I'M GOING TO INTRODUCE AN

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ACTUAL SICKLE CELL DISEASE PATIENT. BUT ARE THERE ANY QUESTIONS?

DR. DULIEGE: VERY IMPRESSIVE. FOR THE PHASE 1/2, PRELIMINARY COMMENTS?

DR. KADYK: I'M NOT AT LIBERTY TO DISCLOSE. THEY ARE ENROLLING. I CAN SAY THAT, BUT I DON'T HAVE ANY RESULTS FOR YOU THAT I CAN SHARE.

OKAY. NO OTHER QUESTIONS? IS MARISSA HERE? I'D LIKE TO INTRODUCE MARISSA COORS, ADRIENNE BELL COORS, ADRIENNE SHAPIRO'S DAUGHTER. MARISSA COORS. I'LL GIVE YOU THE PODIUM.

MS. COORS: HI. HOW ARE YOU? SORRY. EXCUSE ME. LOTION.

MY NAME IS MARISSA COORS. I HAVE SICKLE CELL DISEASE. YOU ALL KNOW MY MOTHER, ADRIENNE SHAPIRO OR ADRIENNE BELL COORS. I'M HERE. I EXIST. I'M THE ONE SHE'S ALWAYS TALKING ABOUT. SHE'S ALSO A MEMBER OF TWO OF YOUR CLINICAL ADVISORY PANELS. AND I REALLY JUST WANT SO SAY, FIRST OF ALL, THANK YOU. THANK YOU FOR SUPPORTING AND FUNDING AND JUST EVEN THINKING ABOUT SICKLE CELL. THAT JUST STARTS OFF. THANKS FOR THAT. IT'S A LITTLE THING, THE THINKING ABOUT US PART. THE FUNDING IS A MASSIVE THING. BUT AS SOMEONE WHO'S LIVED WITH THIS DISEASE 41 YEARS, IT'S NOT BEEN UNTIL THE LAST THREE THAT

WHEN I TELL SOMEBODY I HAVE SICKLE CELL, THAT, A, THEY EVEN KNOW THAT IT EXISTS, LET ALONE HAVE THEIR FACES LIGHT UP AND SAY, "OH, THAT DISEASE IS VERY IMPORTANT." AND I BELIEVE IT'S ALL BECAUSE OF THE WORK YOU'RE DOING IN THIS ROOM.

THAT'S NOT MY SPEECH. THAT WAS JUST SOMETHING I WANTED TO RELAY. TO MY SPEECH.

LIKE I SAID, I'M 41 YEARS OLD. I'VE HAD SICKLE CELL MY ENTIRE LIFE. I WAS DIAGNOSED AT SIX MONTHS, AND I'VE BEEN HOSPITALIZED SO MANY TIMES THAT MY MEDICAL REPORT IS ACTUALLY 8 GIGABYTES. THAT'S ONLY UP UNTIL THE AGE OF 16. MY DOCTOR HAS OVER A THOUSAND PAGES OF MEDICAL NOTES ON ME. THAT'S ONLY AFTER THE AGE OF 28. SO THAT GIVES YOU AN IDEA OF HOW MANY TIMES I SEE MY DOCTOR AND MY HOSPITAL.

WHEN YOU HAVE SICKLE CELL, YOU DEAL WITH PAIN, A LOT OF PAIN. PAIN COMES IN TWO SEPARATE AVENUES. YOU HAVE CHRONIC PAIN AND YOU HAVE ACUTE PAIN. THE CHRONIC PAIN IS AN EVERYDAY THING. FOR INSTANCE, MY RIGHT KNEE, MY LEFT SHOULDER, MY LOWER BACK, AND I HAVE HEADACHES EVERY SINGLE DAY. RIGHT NOW AS I'M SPEAKING TO YOU. I TAKE MEDICATIONS, OF COURSE, TO MANAGE ALL OF THAT. AND ALL OF THAT IS A RESULT OF A LIFETIME OF CRISIS. THE CRISIS IS AN

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ACUTE PAIN, AND THAT PAIN IS SOMETHING THAT YOU WOULD NOT WISH ON THE DEVIL OR YOUR WORST ENEMY. IT IS A HORRIBLE, DEBILITATING EXPERIENCE THAT AFFECTS YOU, NOT JUST PHYSICALLY, BUT MENTALLY, EMOTIONALLY, AND SPIRITUALLY. AND IT IS SOMETHING THAT YOU CANNOT REALLY PUT INTO WORDS, BUT YOU TRY.

IT'S THAT PAIN THAT WE DEAL WITH THE MOST. IT'S THAT PAIN THAT WE HAVE TO MANAGE, NOT JUST OURSELVES, BUT EVERYONE AROUND US THE MOST. WHEN YOU'RE IN A CRISIS -- WHEN YOU ARE IN A CRISIS AND YOU JUST HAVE SICKLE CELL, YOU'RE DEALING WITH ACUTE AND CHRONIC PAIN, YOU'RE IN A CONSTANT STATE OF LIMBO. JUST PERIOD, YOU'RE IN A CONSTANT STATE OF LIMBO. YOU ARE DEALING WITH SOMETHING THAT IS NOT YOUR FAULT, BUT IS YOUR RESPONSIBILITY. IT'S NOT YOUR FAULT, BUT YOUR RESPONSIBILITY.

SO WHAT ENDS UP HAPPENING IS I'VE BEEN ASKED TO TALK TO YOU ABOUT WHAT HAPPENS WHEN YOU HAVE SICKLE CELL. WHAT DOES IT MEAN? WHAT IT MEANS IS THAT YOU'RE DEALING WITH SOMETHING WITHIN YOUR BODY THAT CAN TAKE CONTROL AT ANY TIME. THE PAIN COMES. YOU DON'T KNOW FOR HOW LONG. YOU DON'T KNOW HOW MUCH PAIN YOU'RE GOING TO BE IN. YOU DON'T KNOW HOW MUCH PAIN YOU CAN DEAL WITH AT HOME BEFORE YOU'RE SENT TO THE HOSPITAL. WHEN YOU GET TO THE

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HOSPITAL, YOU DON'T KNOW WHO'S GOING TO TAKE CARE OF YOU. YOU DON'T KNOW IF THEY'RE GOING TO BELIEVE YOU. YOU DON'T KNOW IF THEY'RE GOING TO GIVE YOU THE MEDICATIONS THAT YOU'RE TELLING THEM THAT YOU NEED, THAT YOUR 31 YEARS OF RECORDS SAY YOU GET AND YOU TOLERATE. YOU HAVE NO IDEA HOW LONG YOU ARE GOING TO WAIT IN THE ER. AND WHEN YOU GET TO THE DOCTORS, AND YOU DO ALL OF THIS TALKING, YOU STILL HAVE NO CLUE WHAT THE RESULT IS. IT'S SCARY.

SO YOU'RE WAITING FOR YOUR PAIN TO COME. WHEN IT GETS TO YOU, YOU'RE TRYING TO FIGURE OUT HOW LONG IT PLANS ON STAYING. WHILE YOU'RE TRYING TO MANAGE IT, YOU'RE WONDERING IF THE PEOPLE YOU'RE SPEAKING TO BELIEVE YOU. IF THEY BELIEVE YOU, DO THEY BELIEVE YOU ENOUGH TO DO SOMETHING ABOUT IT? IF THEY FEEL THAT THEY'RE GOING TO DO SOMETHING ABOUT IT, ARE THEY GOING TO FOLLOW YOUR ADVICE, YOUR RECORDS, YOUR FACTS BECAUSE THIS IS YOUR BODY, YOUR ILLNESS, YOUR LIFE, OR ARE THEY GOING TO DO WHATEVER THEY FEEL LIKE DOING? THEY'RE GOING TO DO THE LATTER NINE TIMES OUT OF TEN, WHATEVER THEY FEEL LIKE DOING. LIMBO.

WHEN YOU GET PAIN MEDICATIONS, OFTENTIMES YOU'RE BEING TOLD WE DON'T DO THIS FOR OTHER PEOPLE. IT'S REALLY NOT SOMETHING THAT WE DO WHEN PATIENTS

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COME AND SEE US. THIS AMOUNT OF MEDICATION YOU ARE ASKING FOR IS NOT SOMETHING I'M COMFORTABLE WITH. OKAY. AND THEN THERE'S A PAUSE. AND THE QUESTION IS ALWAYS WHY ARE YOU UNCOMFORTABLE? I'M THE PATIENT. MY LIFE STOPPED. MY MOTHER IS AT HOME OR IN A MEETING, CRYING, TRYING TO HOLD BACK TEARS. MY SISTER IS TRYING TO GET THROUGH FINALS. I'M A 48-YEAR-OLD WOMAN IN TEARS TRYING TO FIGHT FOR MYSELF ALONE IN A HOSPITAL ROOM, IN A HOSPITAL BED, SURROUNDED BY ALL THESE EDUCATED PEOPLE, ALL OF THESE DOCTORS, ALL OF THESE NURSES, NONE OF WHOM UNDERSTAND WHAT I'M GOING THROUGH.

AND I AM EXPECTED TO TAKE RESPONSIBILITY, NOT JUST FOR MY OWN PERSONAL PAIN, NOT JUST FOR MY BEHAVIOR, BUT I AM EXPECTED TO TAKE RESPONSIBILITY FOR THEIR WORK, MEANING THE PAIN MEDICATION OR THE TREATMENTS THEY WILL OR WILL NOT GIVE ME. I'M EXPECTED TO MANAGE THEIR PERSONALITIES AND HOW THEY WORK WITH EACH OTHER AS WELL AS HOW THEY WORK WITH ME. I'M EXPECTED TO ALWAYS BE NICE, ALWAYS SMILE, ALWAYS BE POISED EVEN THROUGH THE PAIN, AND ALWAYS LEAD EVEN THOUGH I'M CONSTANTLY BEING TOLD YOU'RE NOT A DOCTOR, YOU'RE NOT A NURSE. WHERE IS YOUR TRAINING?

MY LIFE IS MY TRAINING. MY EXPERIENCES

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ARE MY TRAINING. THE FOUR GENERATIONS THAT CAME BEFORE ME IN MY FAMILY, THAT'S MY TRAINING. YET NONE OF THAT IS ENOUGH BECAUSE WHEN YOU HAVE SICKLE CELL AND YOU DEAL WITH PAIN, YOU ARE NOT SEEN AS A PERSON, YOU'RE NOT SEEN AS A PATIENT. ONCE YOU BECOME AN ADULT, YOU KNOW WHAT YOU ARE? DRUG SEEKING LIAR, A DRUG ADDICT. SOMEONE WHO IS NOT WORTHY OF THE MEDICAL TRAINING, THE RESOURCES, AND THE TIME THAT THE MEDICAL STAFF WILL SPEND ON YOU IN A HOSPITAL, IN AN ER, IN A DOCTOR'S OFFICE, AT A CANCER CENTER. YOU'RE NONE OF THOSE THINGS.

AND YET, AND YET, IT'S ALWAYS A CONSTANT CYCLE OF, WELL, WHAT DO THEY USUALLY DO? WHAT DO YOU USUALLY DO WHEN YOU HAVE THIS PROBLEM? THIS PROBLEM, LIKE YOU BROKE YOUR SHOE. WHATEVER. IT'S FRUSTRATING. IT'S UPSETTING. IT'S HURTFUL. AND YET YOU HAVE TO FIND HOPE THAT THE NEXT DOCTOR, THE NEXT NURSE, THE NEXT SOCIAL WORKER, THE NEXT BOARD WILL DO BETTER, WILL LISTEN TO YOU, WILL LISTEN TO THE HUNDREDS OF PEOPLE THAT CAME BEFORE YOU, WILL CARE.

I KNOW I'M SUPPOSED TO REALLY RELAY WHAT IT IS TO SORT OF LIVE WITH THIS THING. I KNOW THAT'S WHAT I'M SUPPOSED TO DO UP HERE, BUT CAN I JUST SAY OFF SCRIPT, THEY ALREADY KNOW, WHEN I WAS

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ABOUT FOUR MY MOTHER TOLD ME, I WOKE UP IN THE MIDDLE OF THE NIGHT. FOR SOME REASON SICKLE CELL PATIENTS ALWAYS GO TO THE HOSPITAL AT TWO IN THE MORNING. DON'T KNOW WHY. TWO IN THE MORNING, NEVER FAILS. 3 A.M., NEVER FAILS. YOU'RE WAKING UP THE WHOLE HOUSE, THE WHOLE NEIGHBORHOOD.

I WAS A LITTLE GIRL, MAYBE ABOUT THREE, FOUR, AND I GOT SICK IN THE MIDDLE OF THE NIGHT, 2 O'CLOCK IN THE MORNING, AND I HAD TO WAKE UP MY MOM. AND MY MOTHER, BEING MY MOTHER, IT WAS A LEARNING MOMENT. DIDN'T MATTER I WAS IN PAIN. I HAD TO LEARN SOMETHING. AND WHAT SHE SAID TO ME WAS, WHEN YOU SAY, "MOMMY, MOMMY, WE HAVE TO GO, WHAT ARE YOU REALLY SAYING?" LIKE WE GOT TO GO. SHE SAYS, "NO. THIS IS WHAT YOU'RE DOING. MOM HAS TO GET UP AT THREE. DO WE GET UP AT THREE IN THE MORNING?" "NO, MA'AM." MOM HAS TO NOW CALL DADDY BECAUSE THEY'RE DIVORCED. SHE'S GOING TO WAKE DADDY UP AT 3 O'CLOCK IN THE MORNING? "NO, MA'AM."

OKAY. "MOM HAS TO THEN MAKE SURE THAT YOU'RE DRESSED, EVERYTHING IS PACKED, HAVE TO CALL YOUR DOCTOR AT THREE IN THE MORNING. DO WE DO THAT?" "NO, MA'AM." OKAY. "AND THEN I HAVE TO CALL MY JOB AND TELL THEM I'M NOT COMING IN TODAY BECAUSE YOU'RE SICK. AND THEN MY JOB HAS TO FIND

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SOMEONE TO REPLACE ME AT 3 O'CLOCK IN THE MORNING. IS THIS NORMAL?" "NO, MA'AM. IT'S NOT NORMAL."

"THEN WE HAVE TO LEAVE OUR HOUSE AND GET IN THE CAR AND DRIVE ALL THE WAY TO THE ER AT 3 O'CLOCK IN THE MORNING. DO WE DRIVE AT 3 O'CLOCK IN THE MORNING?" "NO, MA'AM, WE DO NOT BECAUSE WE'RE IN BED." "WHO DRIVES AT 3 O'CLOCK IN THE MORNING?" "I DON'T KNOW. I'M FOUR. PEOPLE."

AND SHE SAYS, "ONCE WE GET TO THE ER, YOU PUT YOUR NAME DOWN ON A LIST." "YES." "AND WE WAIT." "YES." SHE SAID, "NOW WHILE WE'RE WAITING, WHAT'S HAPPENING?" I'M FOUR. I DON'T KNOW. WE'RE WAITING. "WHILE WE'RE WAITING, THERE ARE ALL THESE DOCTORS AND ALL OF THESE NURSES WHO ARE THERE TO TAKE CARE OF YOU AND THE PEOPLE BEFORE YOU AND THE PEOPLE AFTER YOU. AND HOSPITALS HAVE A FINITE NUMBER OF RESOURCES. DO YOU KNOW WHAT FINITE MEANS?" "NO. I'M FOUR. I DON'T KNOW WHAT THAT MEANS."

SHE SAYS, "IT MEANS THAT THERE IS A LIMITED NUMBER. THERE'S A LIMITED NUMBER OF BEDS. THERE'S A LIMITED NUMBER OF RESOURCES. THERE'S A LIMITED NUMBER OF TIME. AND WHEN YOU PUT YOUR NAME ON THAT LIST, YOU ARE SAYING THAT YOU NEED THOSE RESOURCES, THAT TIME, THAT DOCTOR, THAT NURSE, AND

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THAT SPACE. AND IT ALSO MEANS THAT THE PERSON BEHIND YOU IS BEING FORCED TO WAIT FOR YOU BE TO DONE. SO WHAT YOU'RE DOING IS, WHEN YOU SAY, 'MOM, WE GOT TO GO,' YOU ARE AFFECTING THE LIVES OF AT LEAST TEN PEOPLE, AND YOU DON'T KNOW THEIR NAMES AND YOU'VE NEVER SEEN THEIR FACES. SO YOU HAVE TO MAKE SURE THAT YOU'RE REALLY SICK WHEN YOU SAY THOSE WORDS BECAUSE THIS IS WHAT YOU'RE SETTING INTO MOTION.

"IF YOU'RE NOT REALLY SICK, AND WE'VE DONE ALL OF THAT, IF YOU DON'T REMEMBER ANYTHING ELSE, REMEMBER THE HOUR THAT THAT DOCTOR AND THAT NURSE HAVE SPENT ON YOU IN THE BACK WAS AN HOUR THAT YOU TOOK AWAY FROM THE PERSON WHO SIGNED THEIR NAME AFTER YOU. WHEREAS, IT IS NOT YOUR FAULT THAT YOU ARE SICK, IT IS YOUR RESPONSIBILITY TO TAKE CARE OF ALL THAT AND TO UNDERSTAND THE EFFECTS OF WHAT YOU'RE DOING AND WHAT YOU'RE SAYING HAVE ON THESE PEOPLE THAT YOU KNOW NOTHING ABOUT."

AND THAT'S SOMETHING THAT GOES THROUGH LIFE. AND WHEN YOU'RE IN PAIN, CONSTANT PAIN, THAT THEME CONTINUOUSLY COMES. CAN I GO ON VACATION WITH THE FAMILY BECAUSE IF I GET SICK, AM I GOING TO GO? ARE WE ALL GOING TO COME HOME? I PROBABLY SHOULDN'T GO. CAN I GO FOR THIS JOB? WELL, THIS JOB REQUIRES

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ME TO TRAVEL, THIS JOB REQUIRES ME TO DO X, Y, AND Z. IF I GET SICK, CAN I DO THOSE THINGS? MAYBE NOT, SO I PROBABLY SHOULDN'T GO OUT FOR THAT JOB.

IF I WANT TO STUDY TO BE A DOCTOR, SHOULD I BE A DOCTOR BECAUSE IN ORDER TO BE A DOCTOR, YOU CAN'T BE SICK IF YOU'RE A DOCTOR BECAUSE A DOCTOR TAKES CARE OF SICK PEOPLE. DOESN'T MATTER THAT YOU ARE UP IN A HOSPITAL, IN A DOCTOR'S OFFICE, AND YOU KNOW EXACTLY WHAT THEY DO AND HOW THEY DO IT. PROBABLY NOT. THAT WOULD BE IRRESPONSIBLE OF ME.

DO YOU SEE? CONSTANT STATE OF QUESTIONING CAN I DO IT, HOW CAN I DO IT, WHEN CAN I DO IT? IF I DO THIS, IS IT RESPONSIBLE? YOU ARE CONSTANTLY TAKING RESPONSIBILITY FOR OTHER PEOPLE WHILE TRYING TO KEEP YOURSELF WELL, WHILE TRYING TO GET THE THINGS THAT YOU NEED TO GET DONE, WHILE TRYING TO MAKE SURE THAT YOU'RE STILL HERE ANOTHER DAY. YOU'RE CONSTANTLY IN A STATE OF QUESTION. YOU'RE CONSTANTLY IN A STATE OF JUST WAITING FOR THE OTHER SHOE TO DROP AND TRYING TO FIGURE OUT HOW YOU'RE GOING TO CATCH IT EVEN THOUGH YOU GOT LIKE 17 SHOES OVER HERE IN THIS ARM AND 3,000 PAIR OVER HERE IN THIS HAND, BUT IT DOESN'T MATTER BECAUSE YOU'RE THE ONE WITH THE ILLNESS. YOU'RE THE ONE IN PAIN. YOU'RE THE ONE THAT LIVES WITH THIS THING. YOU'RE

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THE ONE THAT HAS TO GO HOME WITH IT, YOU'RE THE ONE THAT HAS TO GO TO BED WITH IT AND WAKE UP WITH IT IN THE MORNING. AND YOU ARE THE ONLY PERSON THERE TO EFFECTIVELY COMMUNICATE TO WHOMEVER IT IS THAT YOU NEED TO WHAT NEEDS TO BE DONE, AND THEN HOPE THAT THAT PERSON LISTENS.

IT IS ABSOLUTELY DRAINING, EXHAUSTING, AND EMOTIONALLY DEPLETING. AND IT'S NOT SOMETHING THAT YOU PUT OUT THERE BECAUSE IT'S NOT POLITE AND NO ONE WANTS TO HEAR YOU WHINE. AND YOU STAND BEFORE PEOPLE VERY WELL DRESSED AND WELL EDUCATED, AND THEY KNOW YOUR MOM, AND THEY KNOW YOUR FAMILY HISTORY, AND THEY'VE HEARD ALL THESE STORIES ABOUT YOU, BUT YOU'RE NOT SUPPOSED TO SAY THINGS LIKE THAT.

AND I'M SAYING THINGS LIKE THAT TO YOU TODAY. I JUST THINK IT IS IMPORTANT. IT'S IMPORTANT THAT I STAND IN FRONT OF YOU WELL EDUCATED, WELL DRESSED, AND REALLY CUTE SHOES, JUST COMING FROM THE NATIONAL SICKLE CELL CONFERENCE AND BEING IN A CRISIS AS WE STAND IN FRONT OF YOU IN PAIN, LETTING YOU KNOW THIS IS WHAT'S GOING ON INSIDE OF ME. I AM IN LIMBO. I AM EXHAUSTED. AND YOUR FUNDING AND YOUR CLINICAL TRIALS AND THE FACT THAT YOU ACTUALLY ALLOWED ME TO CONTINUE TO SPEAK TO YOU THIS LONG CLEARLY OFF SCRIPT TELLS THAT YOU DO

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CARE, YOU DO GIVE A DAMN. AND WHAT IT WILL DO FOR ALL OF THOSE CHILDREN AND PEOPLE WHO HAVE NOT EVEN BEEN BORN YET AND PEOPLE WHO ARE CURE WORTHY, BECAUSE I'M NOT, IT WILL GIVE THEM A CHANCE AT A LIFE WHERE THEY'RE NOT TAKING RESPONSIBILITY FOR ALL OF THIS STUFF, FOR ALL OF THESE PEOPLE BASED ON AN ILLNESS THAT THEY WERE JUST BORN WITH THAT'S NOT THEIR FAULT. IT MEANS THAT THEY WILL GO OUT AND THEY WILL TRY TO HAVE THAT CAREER, THEY WILL TRY TO HAVE THAT FAMILY, THEY WILL WALK IN THE STREET, AND GET OUT OF BED WITHOUT THREE COATS AND TWO DIFFERENT PAIRS OF SHOES IN THEIR PURSE AND A BOTTLE OF WATER. HOPEFULLY WHEN PEOPLE COME UP TO THEM AND THEY SAY, "I MET YOUR MOM AND SHE TALKED TO ME ABOUT YOU," SHE'S NOT TALKING ABOUT THE FACT THAT YOU'VE BEEN IN THE HOSPITAL FOR THREE YEARS OF THE LAST FIVE. I'M DONE.

(APPLAUSE.)

MS. COORS: NO QUESTIONS?

DR. DULIEGE: THANK OF VERY MUCH. THANK YOU FOR THIS WONDERFUL TESTIMONY AND VERY MOVING. WE REALLY APPRECIATE IT.

MS. COORS: THANK YOU FOR LISTENING. I DIDN'T MEAN TO JUST RUN OFF. IT'S JUST USUALLY I HEAR MY MOM START TALKING IN THE BACK, AND THAT

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TELLS ME THAT I NEED TO SIT DOWN NOW. SO I DIDN'T MEAN TO RUN AWAY FROM YOU IF YOU DID HAVE QUESTIONS, BUT I THINK WE'RE HERE, SO, YOU KNOW. YOU HAVE OUR NUMBER. OKAY. HAVE A GOOD DAY. THANK YOU.

(APPLAUSE.)

CHAIRMAN THOMAS: SO ON THE HEELS OF THAT, DO WE HAVE ANY GENERAL PUBLIC COMMENT?

DR. PRIETO: I'D JUST LIKE TO MAKE A COMMENT ABOUT THAT BECAUSE SOMEONE SAID EARLIER TODAY THAT THIS IS NOT A HUGE DISEASE IN TERMS OF THE NUMBERS, BUT I REMEMBER BERT LUBIN, BEFORE HE JOINED THE BOARD, COMING TO TALK TO US ABOUT IT AND LAYING OUT THE POTENTIAL FOR A STEM CELL CURE FOR SICKLE CELL DISEASE. IN JUST KNOWING WHAT I KNOW ABOUT THIS DISEASE AND HOW INADEQUATE OUR CURRENT TREATMENT FOR THIS IS, I REALLY THOUGHT THIS WOULD BE HUGE. AND IF WE ARE A PART OF CURING THIS DISEASE, OR HOWEVER MANY THOUSANDS OF PEOPLE, IT REALLY WILL BE HUGE.

DR. LUBIN: SO THANK YOU FOR INVITING THEM. I WANTED TO SAY SOMETHING, BUT I WANTED TO WAIT TILL THE FAMILY WENT BECAUSE THE BIGGEST GAP THAT WE HAVE IS TAKING CARE OF ADULTS WITH SICKLE CELL. WE HAVE A LOT OF PEDIATRICIANS THAT DO A REALLY GOOD JOB; BUT WHEN THEY TRANSITION TO ADULT,

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THERE'S VERY FEW DOCTORS THAT ARE WILLING TO TAKE CARE OF THOSE. THERE'S NO MONEY IN IT. IT TAKES A LOT OF TIME. AND THE POINT SHE MADE ABOUT PAIN MANAGEMENT IS PATHETIC. I MEAN EMERGENCY ROOM DOCTORS DON'T KNOW WHAT TO DO AND DON'T LISTEN TO THE PATIENTS. AND SHE WAS SO PASSIONATE ABOUT IT.

NIH IS INVESTING A LOT IN EDUCATION TO TRY TO CHANGE THIS, BUT I THINK WHAT WE'RE TALKING ABOUT HERE AT CIRM IS SOMETHING THAT'S GOING TO BE AVAILABLE BEFORE WE EVEN GET TO THAT STAGE. WE'RE GOING TO PREVENT HAVING TO WORRY ABOUT GOING TO THE EMERGENCY ROOM AND WHETHER THE DOCTOR BELIEVES YOU OR NOT. AND WHAT SHE SAID IS ABSOLUTELY ACCURATE, THAT DOCTORS DO NOT BELIEVE WHAT THE PATIENT'S ASKING FOR FOR PAIN. AND IT'S TRAGIC. IT IS REALLY TRAGIC IN THIS SOPHISTICATED SOCIETY THAT THAT HAPPENS.

BECAUSE OF A NUMBER OF THINGS, RACIAL AND OTHER FACTORS, THIS POPULATION HAS NOT BEEN SERVED. AND WHEN YOU LOOK AT FUNDS THAT HAVE BEEN ALLOCATED FOR DIFFERENT THINGS, SICKLE CELL HAS NEVER BEEN HIGH ON THE LIST BECAUSE THERE AREN'T PUBLIC ADVOCACY GROUPS. THE MOM HERE, SHE'S PHENOMENAL, BUT SHE'S ONE; WHEREAS, OTHER DISEASES, CYSTIC FIBROSIS AND OTHERS, THERE'S ENORMOUS AMOUNT OF

EFFORT TO RAISE MONEY. SO WONDERFUL THAT CIRM IS TAKING A STANCE IN THIS, AND A PARTNERSHIP WITH NHLBI REALLY SAYS, AS WE HEAR MANY TIMES, THAT CALIFORNIA IS GOING TO BE A LEADER. YOU ARE GOING TO BE THE LEADER IN CHANGING THIS FOR THE NATION AND MAYBE THE WORLD. SO I THINK IT'S REALLY A WONDERFUL OPPORTUNITY FOR US. AND I WANT TO THANK ALL OF YOU BECAUSE I THINK IT'S SO IMPORTANT, AND I THINK WE CAN DO IT.

MR. TORRES: WE THANK YOU FOR YOUR LEADERSHIP.

(APPLAUSE.)

CHAIRMAN THOMAS: OKAY. WELL, ON THAT HIGH NOTE AND SEEING NO PUBLIC COMMENT, THANK YOU, EVERYBODY, FOR A GREAT MEETING. I DO WANT TO REMIND YOU THAT WE NEED TO GET A QUORUM FOR THAT NOVEMBER 15TH APPLICATION REVIEW SUBCOMMITTEE. WE'LL CALL THAT THE OS STEWARD MEETING. AND ON THAT NOTE, HAVE A GREAT REST OF THE MONTH.

I WOULD LIKE TO PERSONALLY THANK MR. ROWLETT, MR. JUELSGAARD, DR. SAMBRANO, AND OTHERS FOR YOUR CONTINUED BEST WISHES FOR THE DODGERS AS THEY CONTINUE TOWARDS THE WORLD SERIES. WE STAND ADJOURNED. THANK YOU VERY MUCH.

(THE MEETING WAS THEN ADJOURNED AT 1:45 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 AT THE LOCATION INDICATED BELOW

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
1999 HARRISON STREET, SUITE 1650
OAKLAND, CALIFORNIA
ON
OCTOBER 11, 2018

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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