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

REGULAR MEETING

LOCATION: AS LISTED ON THE AGENDA

DATE: OCTOBER 11, 2018
2 P.M.

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

FILE NO.: 2018-13B

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OCTOBER 11, 2018; 2 P.M.

MR. SHEEHY: GOOD AFTERNOON, EVERYONE. THANK YOU FOR BEING AVAILABLE TODAY. I'D LIKE TO CALL THE MEETING OF THE SCIENCE SUBCOMMITTEE OF THE ICOC TO ORDER, PLEASE. COULD YOU CALL THE ROLL.

MS. BONNEVILLE: DEBORAH DEAS.

DR. DEAS: HERE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: HERE.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: HERE.

MS. BONNEVILLE: BERT LUBIN.

DR. LUBIN: HERE.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: HERE.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: HERE.

MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: HERE.

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MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: HERE.

MR. SHEEHY: SO WE'LL START OFF WITH A PRESENTATION BY DR. MILLAN THAT WILL LINE OUT THE BUDGET, AND ALSO EMBEDDED IN THAT IS A DISCUSSION OF AN MOU WITH THE NATIONAL HEART, LUNG AND BLOOD INSTITUTE CURE SICKLE CELL INITIATIVE AND THE RELATIONSHIP WITH CIRM.

DR. MILLAN: THANK YOU VERY MUCH. THANK YOU VERY MUCH, MR. SHEEHY. THANK YOU FOR THE SCIENCE SUBCOMMITTEE AND MEMBERS OF THE PUBLIC AND COLLEAGUES.

SO I'LL JUST BE ABOUT GIVING A BRIEF UPDATE AND JUST A BACKGROUND TO SET UP THE DISCUSSION ABOUT THE PROPOSED BUDGET FOR 2019 AS WELL AS THE SICKLE CELL CURE INITIATIVE AND OUR MOU, MEMORANDUM OF UNDERSTANDING, PARTNERSHIP WITH THE NIH ON CURE SICKLE CELL AS WELL AS CONCEPT CHANGES THAT WILL BE PRESENTED LATER THAT WILL SUPPORT BOTH OUR PLANS FOR THE BUDGET AS WELL AS THE INITIATIVE.

OUR MISSION IS TO CONTINUE TO ACCELERATE STEM CELL TREATMENTS TO PATIENTS WITH UNMET MEDICAL NEEDS. THAT DOESN'T CHANGE. THAT DRIVES HOW WE OPERATE. AND NEXT SLIDE PLEASE.

AND IN SERVICE OF THIS MISSION, YOU ARE

ALL AWARE THAT WE LAUNCHED A FIVE-YEAR STRATEGIC PLAN BEGINNING IN 2016 WITH THE OVERARCHING GOAL OF ACCELERATING DEVELOPMENT OF PROMISING SCIENCE INTO POTENTIAL THERAPEUTICS FOR PATIENTS WITH UNMET MEDICAL NEEDS.

JUST A VERY BRIEF OVERVIEW OF THE BIG SIX OF THE STRATEGIC PLAN IS TO BRING 50 NEW CANDIDATES INTO DEVELOPMENT, INCREASE THE PROGRESSION OF PROJECTS GOING FROM ONE STAGE TO THE NEXT BY 50 PERCENT. AND THAT'S A NEW REGULATORY PARADIGM THAT ACCELERATES THESE PROGRAMS. REDUCE THE TIME IT TAKES TO TRANSLATE THE SCIENCE INTO CLINICAL PROGRAMS, ADD 50 NEW CLINICAL TRIALS, BRINGING US TO A TOTAL OF 67 CLINICAL TRIALS FOR CIRM, AND PARTNER AT LEAST HALF OF OUR UNPARTNERED PROGRAMS SO THEY CAN BE TAKEN TO COMMERCIALIZATION.

THOUGHT IT WOULD BE A GOOD IDEA, GIVEN WHERE WE ARE IN OUR FUNDING LIFE, THAT WE TALK ABOUT WHAT THE GENERAL INVESTMENT HAS BEEN INTO OUR FIVE PILLARS OF INVESTMENT IN CIRM. SO FOR INFRASTRUCTURE, APPROXIMATELY 480 MILLION INFRASTRUCTURE DOLLARS THAT INCLUDED EARLY-ON BUILDING INFRASTRUCTURE AND LATER ON PROGRAMMATIC INFRASTRUCTURE, SUCH AS THE ALPHA CLINICS NETWORK AND OUR PARTNERSHIP TO CREATE STEM CELL-SPECIFIC

CLINICAL RESEARCH ORGANIZATIONS.

ALMOST 900 MILLION, 898 MILLION, INTO THE DISCOVERY PROGRAMS, 334 MILLION INTO TRANSLATIONAL STAGE PROGRAMS. TO REMIND EVERYBODY, TRANSLATIONAL STAGE PROGRAMS ARE MEANT TO DO THE SCIENCE TO DEVELOP THE PACKAGE TO GO TO THE FDA TO GET INPUT IN TERMS OF WHAT IT WOULD TAKE TO GET AN IND IN ORDER TO GET INTO TRIAL. 632 MILLION SO FAR, THIS HAS BEEN EXPANDING ESPECIALLY OVER THE PAST THREE YEARS AS WE INCREASE OUR CLINICAL PROGRAMS, $632 MILLION INTO THE CLINICAL PROGRAM, AND $219 MILLION INTO EDUCATION PROGRAMS.

AS YOU ALL WILL REMEMBER, NOVEMBER 2017 AND THEN AGAIN IN JANUARY OF -- NO. I THINK IT WAS IN NOVEMBER OF 2016 WE PRESENTED THIS TRANSITION PLAN; IS THAT CORRECT? 2017. I'M SORRY. WE PRESENTED TO THE BOARD A TRANSITION PLAN TO ACCOUNT FOR WHAT WE WOULD DO IN TERMS OF OUR PROGRAMS AND OUR BUDGET IN THE REMAINING YEARS OF CIRM WITH THE REMAINING ADMINISTRATIVE AND RESEARCH BUDGET.

AND THE GUIDING PRINCIPLES AROUND THAT IS THAT WE WOULD CONTINUE TO PUSH HARD AND ACHIEVE THE FIVE-YEAR STRATEGIC PLAN TO THE BEST OF OUR ABILITY WITHIN BUDGET, THAT WE WOULD MAINTAIN THE CRITICAL LEVEL OF PERSONNEL AND QUALITY OF PERSONNEL,

QUALIFIED PERSONNEL, SO THAT WE ARE ABLE TO EXECUTE ON THIS PLAN WHILE MAINTAINING OPERATIONAL EXCELLENCE, AND THAT WE FELT IT WAS ESSENTIAL TO PRESERVE CIRM'S VALUE PROPOSITION. THE VALUE PROPOSITION BEING TO INVEST IN PROGRAMS AND OTHER PROMISING PROGRAMS THAT COULD LEAD TO THERAPIES WHEN OTHER INVESTORS OR AGENCIES ARE NOT TYPICALLY READY OR ARE NOT ABLE TO FUND THOSE TYPE OF PROGRAMS IN ORDER TO INCREASE THE PROBABILITY OF GETTING TO PATIENTS.

THE OUTPUT OF THIS, A SURROGATE MEASURE FOR THIS, IS NUMBER OF PROGRAMS THAT GET INTO CLINICAL TRIALS. AND SO FAR WE HAVE FUNDED 49 NEW CLINICAL TRIALS. THAT BRINGS US UP TO A TOTAL OF 56, AND IT IS BROAD IN TERMS OF DISEASE INDICATIONS WITH DIVERSE THERAPEUTIC APPROACHES. WE WON'T GO INTO THESE SPECIFIC PROGRAMS TODAY, BUT SUFFICE IT TO SAY THAT IT'S A ROBUST PORTFOLIO OF TRIALS.

SO IN 2018 WANTED TO GIVE AN UPDATE OF WHERE WE ARE IN TERMS OF OUR RESEARCH SPEND FOR 2018. IN THE SECOND COLUMN, IT TALKS ABOUT THE 2018 ALLOCATION FOR THE FOUR PROGRAMS AS LISTED ABOVE. I'LL JUST GO AHEAD AND SUMMARIZE THAT: 130 MILLION FOR CLINICAL, 30 MILLION FOR TRANSLATION, 10 MILLION FOR DISCOVERY, AND 750,000 FOR EDUCATION.

THE ESTIMATED 2018 YEAR-END SPEND IS LISTED ON THE THIRD COLUMN, LEAVING US A LITTLE RESIDUAL IN CLINICAL. WE WILL HAVE PROBABLY SPENT MOST OF THE TRANSLATION OR ACTUALLY 28 MILLION OF THE 30 MILLION TRANSLATION, ALL OF THE DISCOVERY AFTER TODAY'S EARLIER MEETING, AND 150,000 LEFT IN EDUCATION. WHAT THAT BROUGHT US OR IS BRINGING IS SEVEN NEW CLINICAL TRIALS, SIX PRECLINICAL PROGRAMS LEADING TO CLINICAL TRIALS, SEVEN NEW CANDIDATES, AND SEVEN PROJECTS IN DISCOVERY.

SO WHERE ARE WE IN TERMS OF THE 2018 YEAR-END BUDGET? AS OF JANUARY 2018, THE BEGINNING OF THIS YEAR, THIS CALENDAR YEAR, WE HAD A COMMITTED DISBURSED AMOUNT OF $2.48 BILLION, LEAVING US WITH A $269 MILLION RESEARCH FUNDING ALLOCATION THAT WAS AVAILABLE. WITH THE ESTIMATED 2018 ACTIVITY THAT'S LISTED HERE, RESULTING IN 30, WE ESTIMATE 30 TOTAL AWARDS FOR THE YEAR AND SOME RECOVERY OF UNEXPENDED FUNDS FROM SOME OF THE PROGRAMS. THIS LEAVES US WITH $144 MILLION IN OUR RESEARCH BUCKET.

WE DO ESTIMATE THAT IN THE UPCOMING YEAR IN 2019, BASED ON PREVIOUS YEARS AND BASED ON WHAT'S ACTIVE IN OUR PORTFOLIO, THAT WE WILL RECOVER APPROXIMATELY $30 MILLION IN 2019. HOWEVER, WE DID NOT PUT THAT IN YOUR CURRENT PROPOSAL FOR BUDGET

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ALLOCATION FOR 2019 BECAUSE WE ARE NOT CERTAIN WHEN THESE FUNDS WOULD COME IN AND EXACTLY HOW MUCH THEY WOULD BE.

BASED ON THAT, I'D LIKE TO GO TO THE NEXT SLIDE. THIS JUST SUMMARIZES WHAT WE ESTIMATE WE'LL START THE YEAR OFF IN 2019 IS $144 MILLION IN RESEARCH, AS DISCUSSED, AND $39 MILLION FOR ADMINISTRATION. JUST TO REMIND EVERYBODY, THESE ARE TWO SEPARATE BUCKETS, MEANING THEY'RE BUDGETS THAT CANNOT BE INTERMIXED. THEY'RE USED FOR THE RESPECTIVE PURPOSES ONLY.

SO IN TERMS OF THE 2019 BUDGET, OF THE $144 MILLION IN RESEARCH BUDGET, WHAT WE WILL BE PROPOSING TODAY FOR CONSIDERATION TO THE SUBCOMMITTEE IS THE BUDGET THAT DOES NOT HAVE ANY ALLOCATION FOR DISCOVERY STAGE PROGRAMS, THAT WE NARROW THE ELIGIBILITY FOR TRAN AND CLIN1 PROGRAMS, AND YOU WILL HEAR WHAT THAT WOULD BE FROM DR. SAMBRANO, WHO WILL PRESENT THE PROPOSED PA CHANGES. AND THEN CONSISTENT WITH THE STRATEGIC PLAN OF TARGETING 50 NEW CLINICAL TRIALS TO OPTIMIZE THE BUDGET TO BRING IN CLINICAL PROGRAMS; HOWEVER, WITH THE REMAINING BUDGET, WE STILL WILL NOT BE ABLE TO HIT THE TARGETED 50 NEW CLINICAL TRIALS. WE ESTIMATE THAT AT MOST WE BRING IN 43 TO 45 NEW

CLINICAL TRIALS, BUT THAT WOULD STILL BRING THE TOTAL NUMBER THAT CIRM HAS SUPPORTED TO APPROXIMATELY 60.

WE PROPOSE A SET ASIDE OF $30 MILLION TO FUND THE JOINT CIRM/NHLBI SICKLE CELL CURE INITIATIVE. YOU'LL HEAR ABOUT THAT INITIATIVE A LITTLE BIT MORE BEFORE WE BRING THIS FOR YOUR CONSIDERATION FORMALLY FOR A BUDGET ITEM. THE REASON THAT WE'RE ASKING FOR THIS SET ASIDE IS THAT IN ORDER TO HAVE THIS JOINT PROGRAM EXECUTED, WE WOULD NEED TO HAVE ACCESS TO A RELIABLE BUCKET OF FUNDS. WE PROJECT, BASED ON LANDSCAPE ANALYSIS AND FORECASTING THAT WAS DONE BY THE CIRM AND NIH TEAM, THAT THERE ARE AT LEAST SIX PROGRAMS THAT WE BELIEVE, THAT WE ALREADY KNOW ABOUT AND ADDITIONAL THAT ARE OUT THERE. SO FOR A $30 MILLION CIRM ALLOCATION, THE NIH WOULD MATCH THOSE FUNDS. SO WE THINK THAT WE WOULD AT LEAST BE ABLE TO BRING IN AT LEAST FIVE OR SIX PROGRAMS FOR THE $30 MILLION SET ASIDE.

THE REMAINING ADMINISTRATIVE BUDGET, AS CONSISTENT WITH THE TRANSITION PLAN, WE'LL BE ABLE TO SUPPORT SUFFICIENT STAFFING AND A PLAN THAT WOULD MAKE SURE THAT ALL CIRM AWARDS WOULD BE MANAGED REGARDLESS OF WHETHER OR NOT THERE'S ADDITIONAL

FUNDING BEYOND 2020 THAT COMES INTO THIS AGENCY.

THE PROPOSED BUDGET, WHICH WE WILL NOT ASK FOR A MOTION OR ACTION AT THIS TIME, IS JUST SHOWN ON THIS CHART. WE'RE PROPOSING A $93 MILLION BUDGET FOR CLIN1 AND 2 AND A SPECIFIC SET ASIDE FOR CLIN1 AND 2 FOR THE SICKLE CELL DISEASE JOINT NHLBI/CIRM INITIATIVE.

WE'RE PROPOSING $20 MILLION FOR THE TRANSLATIONAL STAGE PROGRAMS. AS MUCH AS WE WOULD LIKE TO BE ABLE TO HAVE A BUDGET FOR DISC, WE ARE RECOMMENDING THAT WE PUT THE ALLOCATIONS TOWARDS THE LATER STAGE PROGRAMS. AND THE 600,000 THAT'S LEFT FOR EDUCATION IS FOR THE SPARK, BRIDGES PROGRAM, AND THE ALPHA CLINIC SYMPOSIUM. THEY'RE PROGRAMS THAT WE'VE ALREADY COMMITTED TO.

AND SO LIKE TO LEAVE THIS OPEN -- TURN IT BACK TO MR. SHEEHY FOR COMMENTS, QUESTIONS, AND THEN WE WILL HAVE THE DESCRIPTION OF THE NHLBI MOU.

MR. SHEEHY: I'M WONDERING IF IT'S OKAY WITH THE OTHER MEMBERS THAT WE GO AHEAD AND TALK ABOUT THE MOU SINCE THAT'S PART OF THE BUDGET AND THEN COME BACK AND HAVE A GENERAL BUDGET DISCUSSION.

DR. DEAS: THAT'S FINE.

MR. THOMPSON: THANK YOU, MR. SHEEHY. THIS IS GABRIEL THOMPSON. I'M THE VICE PRESIDENT OF

GRANTS AND OPERATIONS AT CIRM. AND I'M HERE TO DESCRIBE GENERALLY HOW THE CURE SICKLE CELL CO-FUNDING INITIATIVE WILL WORK.

AND SO, AS YOU GUYS KNOW, CIRM AND NHLBI SIGNED AN MOU IN LATE JUNE TO AGREE TO A CO-FUNDING INITIATIVE. AND THE PURPOSE OF THE INITIATIVE IS ACCELERATE IMPLEMENTATION OF ACCESSIBLE CURES FOR SICKLE CELL DISEASE WITH A GOAL OF FINDING A CURE WITHIN FIVE TO TEN YEARS. SO WE THINK THERE'S AN ALIGNMENT OF PURPOSE HERE GIVEN THE ACCELERATION AND PRIORITY FOR THIS SPECIFIC DISEASE.

WE BELIEVE THIS PARTICULAR CO-FUNDING INITIATIVE IS QUITE UNIQUE IN CONTRAST TO OTHER COLLABORATIVE FUNDING PARTNERSHIPS WE'VE HAD IN THAT UNDER THIS INITIATIVE, NHLBI IS PROVIDING FUNDS TO CIRM AND ADOPTING OUR CLINICAL STAGE PROGRAM. SO THIS INCLUDES OUR CLIN1, OUR CLIN2, AND OUR CLIN3 PROGRAMS.

SO WHAT THIS MEANS IS THAT THERE WILL BE -- IT UTILIZES THE CIRM CLINICAL PROCESS. SO THERE WILL BE A SINGLE CIRM APPLICATION THAT APPLICANTS WILL APPLY FOR. THERE WILL BE A SINGLE SCIENTIFIC PEER REVIEW, WHICH WILL BE PART OF CIRM'S GRANTS WORKING GROUP. AND THEN THERE WILL BE SINGLE CIRM AWARD THAT THE AWARDEE WILL MANAGE. SO THIS,

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WE THINK, IS A HIGHLY UNIQUE SITUATION AND IS REALLY VALIDATING A LOT OF THE ACCELERATING ENGINE WE PUT INTO PLACE YEARS AGO.

WE STILL MAINTAIN THE ACCELERATION BY HAVING FUNDING DECISIONS IN AS LITTLE AS 85 DAYS. OBVIOUSLY, THE FUNDING NHLBI PROVIDES WILL ALLOW US TO LEVERAGE THAT FUNDING WITH OUR OWN IN ORDER TO SUPPORT MORE PROJECTS THAN WE COULD IF WE WERE ACTING ALONE.

THERE'S ALSO A DATA SHARING COMPONENT UNDER THE INITIATIVE THAT WE ARE WORKING OUT IN DETAILS THAT WE'LL SHARE LATER ON.

SO JUST TO BRIEFLY GO OVER, WE HAVE ATTEMPTED TO TRY TO CHANGE AS LITTLE AS POSSIBLE ABOUT OUR CLIN PROGRAM IN SUPPORT OF THIS INITIATIVE; BUT TO LIST OFF THE FEW CHANGES THAT WE ARE MAKING TO THE PROGRAM AND ALSO WILL BE DESCRIBED LATER ON IN THE CONCEPT PLAN CHANGES, BUT WE OBVIOUSLY NEED TO INFORM APPLICANTS THAT THEIR APPLICATION MATERIALS WILL BE SHARED WITH NHLBI REPRESENTATIVES. UNDER THE INITIATIVE, AND THAT'S SPELLED OUT IN THE MOU, AWARDEES ARE GOING TO BE REQUIRED TO COMPLY WITH NHLBI DATA SAFETY AND MONITORING AS WELL AS DATA SHARING POLICIES.

THE INITIATIVE IS UNIQUE IN THAT WE ARE

GOING TO ALLOW NON-CALIFORNIA ORGANIZATIONS TO APPLY WHO ARE REQUESTING THEIR CIRM UNALLOWABLE COSTS TO BE COVERED BY NHLBI FUNDS.

AND THEN FINALLY ALL SICKLE CELL APPLICATIONS WILL BE SUBJECT TO THIS REVISED PROGRAM SO THAT WE ENSURE THAT WE ARE GETTING THE LEVERAGE WE WOULD LIKE FROM THESE PROGRAMS.

JUST BRIEFLY, AS FAR AS THE PROCESS, AGAIN, WE HAVE ATTEMPTED TO KIND OF MAINTAIN THE VERY RAPID PROCESS FOR TAKING APPLICATIONS IN AND REVIEWING FOR ELIGIBILITY, GOING TO PEER REVIEW, AND THEN MAKING A FUNDING DECISION.

WHEN APPLICATIONS COME IN UNDER THIS PROGRAM, CIRM WILL CONDUCT ELIGIBILITY REVIEW AND NHLBI REPRESENTATIVES WILL BE GIVEN ACCESS TO THE APPLICATIONS VIA OUR GRANTS MANAGEMENT SYSTEM.

THE APPLICATIONS, ONCE THEY PASS ELIGIBILITY, WILL GO TO THE GRANTS WORKING GROUP FOR SCIENTIFIC PEER REVIEW IN WHICH NHLBI CAN ATTEND THOSE MEETINGS.

AND THEN NHLBI HAS AGREED TO MAKE ITS FUNDING DECISION WITHIN TEN DAYS OF THE GWG SCIENTIFIC PEER REVIEW MEETING. AND THEN WE WILL TAKE BOTH THE SCIENTIFIC PEER REVIEW AS WE NORMALLY DO ALONG WITH NHLBI'S FUNDING DECISION AND DOLLAR

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COMMITMENT TO THE ICOC APPLICATION REVIEW SUBCOMMITTEE FOR A FINAL FUNDING DECISION. WE THINK WE CAN STILL DO THIS AND MAINTAIN OUR PROCESS TIME TO GET APPLICATIONS -- A FUNDING DECISION IN AS LITTLE AS 85 DAYS.

FINALLY, ON THE KIND OF AWARD MANAGEMENT. SO AFTER FUNDING APPROVAL, IF APPLICATIONS ARE RECOMMENDED FOR FUNDING, NHLBI FUNDS WILL COME TO CIRM VIA WHAT IS CALLED THE OTHER TRANSACTIONAL AUTHORITY. AND THIS IS WHAT -- WHAT THAT BASICALLY MEANS IS THIS IS HOW NIH GETS FUNDS TO OTHER ENTITIES THAT AREN'T SUBJECT TO THE NORMAL SET OF FEDERAL GRANTS POLICIES AND REGULATIONS.

CIRM WILL BE ISSUING THE NOTICE OF AWARD TO THE AWARDEE FOR BOTH THE CIRM AND NHLBI FUNDS. THE AWARDEE WILL WORK WITH CIRM TO SUBMIT THE NORMAL PROGRESS AND FINANCIAL REPORTS. THAT WILL ALSO BE SHARED WITH NHLBI VIA OUR GRANTS MANAGEMENT SYSTEM. AND NHLBI REPRESENTATIVES CAN BE APPOINTED TO OUR CLINICAL ADVISORY PANELS AS WELL. AND WE'RE EXCITED TO HAVE THEM BE A PART OF THAT PROCESS THAT WE THINK IS REALLY HELPING GUIDE THESE PROGRAMS FORWARD. AND THEN CIRM DOES RETAIN THE ABILITY TO SUSPEND OR TERMINATE THE AWARDS, AND WE WOULD OBVIOUSLY BE IN CONTACT WITH NHLBI IF WE EVER NEEDED TO TAKE A

DECISION TO SUSPEND OR TERMINATE AN AWARD.

SO THOSE WERE THE KIND OF GENERAL COMMENTS. WE ARE WORKING TOWARD AN AGREEMENT WITH NHLBI THAT WE HOPE TO HAVE EXECUTED BY THE THANKSGIVING HOLIDAY. AND IF THAT'S POSSIBLE, THEN WE WOULD START ACCEPTING APPLICATIONS UNDER THIS PROGRAM FOR THE DECEMBER 31ST OR WHATEVER THAT LAST WORKING DAY OF DECEMBER IS, THE FIRST DEADLINE WE WOULD RECEIVE APPLICATIONS UNDER THIS PROGRAM.

SO I'M HAPPY TO TAKE COMMENTS OR QUESTIONS ABOUT THE PROCESS IN GENERAL OR ANY OTHER QUESTIONS YOU HAVE ABOUT THE INITIATIVE.

DR. DEAS: I DO HAVE A QUESTION. MAYBE I MISSED SOMETHING BECAUSE IT SEEMS PRETTY STRAIGHTFORWARD, AND I THINK THIS IS A GREAT COLLABORATION. I KNOW THAT WE'RE SETTING ASIDE 30 MILLION FOR THE AREA OF SICKLE CELL DISEASE. AND BY HAVING THIS MOU OF A COLLABORATION, WHAT AMOUNT OF FUNDS WILL BE COMING FROM THE NATIONAL INSTITUTE, NHLBI? DID YOU SAY THAT?

DR. MILLAN: DR. DEAS, IT'S MARIA MILLAN. I'M GOING TO GO AHEAD AND START THE RESPONSE TO THAT QUESTION, AND GABE CAN CHIME IN.

WHERE WE ARE NOW IS THAT WE'RE PROPOSING THAT THE NHLBI SHARE IN THE CIRM COSTS. SO WHATEVER

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OUR AWARD WOULD BE, THEY WOULD CO-FUND THAT. SO WE ARE PROPOSING A CERTAIN PERCENTAGE OF THAT, AND THAT'S WHAT GABE IS TALKING ABOUT RIGHT NOW. WE HAVE A GENERAL BUY-IN TO THAT AT THIS POINT.

IN ADDITION TO SHARING THE CIRM COSTS, THE NHLBI IS OPEN TO ALSO FUNDING ADDITIONAL COSTS THAT CIRM WOULDN'T OTHERWISE FUND BECAUSE EITHER THEY'RE OUT-OF-STATE OR WHAT HAVE YOU.

DR. DEAS: OKAY.

DR. MILLAN: WE ACTUALLY GET LEVERAGE OFF THIS SO THAT THEIR CONTRIBUTION WILL OFFSET CIRM'S COST FOR THE LIFE OF THE PROJECT.

DR. DEAS: OKAY. I GOT IT. ALL RIGHT. THIS IS GREAT. THANK YOU.

DR. MILLAN: YOU'RE WELCOME.

DR. LUBIN: I'D LIKE TO ASK A QUESTION. FOR THE NIH COMPONENT -- FIRST OF ALL, THIS IS TERRIFIC. FOR THE NIH COMPONENT, DOES IT STILL GO THROUGH STUDY SECTION AND COUNCIL BEFORE IT GETS TO US?

MR. THOMPSON: NO, NOT AT ALL. SO THE NHLBI CURE SICKLE CELL INITIATIVE WILL HAVE AN EXECUTIVE COMMITTEE THAT WILL MAKE THE FUNDING DECISIONS WITHIN TEN DAYS OF OUR GRANTS WORKING GROUP. AND THAT IS THE EXTENT OF THEIR

DECISION-MAKING. NO NIH PEER REVIEW PROCESS INVOLVED.

DR. LUBIN: SO IF I WERE IN DELAWARE AND DOING WORK ON STEM CELLS IN SICKLE CELL, WOULD I APPLY TO THE NIH OR APPLY TO CIRM?

MR. THOMPSON: IF YOU HAD SOME OF YOUR PROJECT ENROLLING PATIENTS -- IF IT WAS A CLINICAL TRIAL AND YOU HAD PATIENTS YOU WERE ENROLLING IN CALIFORNIA OR YOU HAD ACTIVITIES THAT YOU WERE CONDUCTING IN CALIFORNIA, THEN IT WOULD BE BENEFICIAL TO APPLY UNDER THIS PROGRAM.

DR. MILLAN: I JUST WANTED TO SAY THAT, IN ADDITION, WE HAD THIS CONVERSATION WITH THEM. AND THE INTENT IS ANY NEW PROGRAMS COMING IN FOR CELL AND GENE THERAPY FOR CURE SICKLE CELL WOULD COME IN THROUGH THIS INITIATIVE. WE JUST HAD THE CONVERSATION WITH THEM AND THE ADVANTAGES OF CONSOLIDATING THAT HALFWAY.

THERE MAY BE OTHER TYPES OF PROGRAMS THAT MAY BE SMALL MOLECULE OR OTHER KIND OF TRADITIONAL APPROACHES THAT MAY STILL GO THROUGH THE NIH. BUT THE FOCUS REALLY IS, AND THAT'S THE REASON FOR THE PARTNERSHIP WITH CIRM, IS THE BELIEF THAT THE CURE WILL COME FROM A CELL/GENE THERAPY.

DR. LUBIN: SOUNDS GOOD. THANK YOU.

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DR. MILLAN: THANK YOU.

MR. SHEEHY: CHAIRMAN THOMAS.

CHAIRMAN THOMAS: ONE QUICK THING. I JUST WANT TO ECHO THE COMMENTS THAT WE HAD IN JUNE WHEN YOU FIRST DESCRIBED THIS, MARIA, TO SAY AND REITERATE WHAT A TREMENDOUSLY EXCITING PROGRAM THIS IS, NOT ONLY IN TERMS OF HOW IT'S GOING TO ADVANCE THERAPIES AND CURES FOR THIS DISEASE, WHICH IS THE PRINCIPAL GOAL, BUT THE FACT THAT PARTNERSHIP SERVES AS A GREAT TEMPLATE FOR HOW WE CAN PROCEED WITH NIH IN CONNECTION WITH A NUMBER OF DIFFERENT OTHER PROJECTS, ASSUMING WE GET OUR FUNDING GOING AND RENEWED, ETC., BUT IT IS A TERRIFIC EXAMPLE. I JUST WANTED TO COMMEND YOU FOR GETTING THIS IN PLACE. I THINK IT'S JUST A GREAT IDEA.

DR. MILLAN: THANK YOU. IT'S A TEAM EFFORT, REALLY A HUGE AMOUNT OF WORK FROM GABE'S TEAM, ABLA'S TEAM, AND OTHERS IN THE ORGANIZATION TO WORK THIS THROUGH.

DR. JUELSGAARD: COUPLE OF QUESTIONS. THE FIRST RELATES TO THE PERIOD OF TIME OVER WHICH YOU MIGHT REASONABLY EXPECT TO SPEND $30 MILLION. DO YOU EXPECT TO SPEND THAT OR SPEND -- LET ME NOT SAY SPEND, BUT COMMIT THAT AMOUNT OF MONEY. DO YOU EXPECT THOSE COMMITMENTS IN ONE YEAR, TWO YEARS,

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THREE YEARS? WHAT'S SORT OF THE RUN RATE THAT YOU'RE SEEING?

DR. MILLAN: SO WHAT WE'RE ASKING FOR IS A ONETIME ALLOCATION BECAUSE IF ALL SIX OF THESE PROGRAMS COME IN NEXT YEAR, WE'D WANT TO BE ABLE TO GO AHEAD AND GET THESE GOING AND NOT HOLD IT UP. BUT WHAT WE ANTICIPATE DOING IS THAT THIS WOULD BE OVER THE COURSE, PROBABLY, OF TWO YEARS, AT LEAST BE AVAILABLE SO THAT IN THE SECOND YEAR, IF THEY'RE NOT QUITE READY IN 2019, THAT THE FUNDS WOULD BE THERE TO FUND THIS CO-FUNDING SCHEME FOR 2020.

DR. JUELSGAARD: GREAT. SECOND QUESTION THEN. THE $30 MILLION IS WHAT WE ARE SETTING ASIDE. I'LL USE MY TERMINOLOGY FOR A MOMENT. ARE WE GOING TO ADVISE THE NIH NATIONAL HEART, LUNG AND BLOOD INSTITUTE THAT WE'RE SETTING ASIDE $30 MILLION? IF SO, DO THEY SEE THAT AS A FIRM COMMITMENT ON OUR PART TO SPEND THAT AMOUNT OF MONEY?

DR. MILLAN: WE HAVE INFORMED THEM THAT -- WE'VE HAD SOME REALLY INTENSE PLANNING SESSIONS. AND THIS $30 MILLION HAS BEEN A GREAT STIMULUS TO GET THINGS GOING ON THE OTHER SIDE IN TERMS OF -- SO I THINK THE FACT THAT THEY KNOW THAT WE'RE READY TO GO IS GETTING THEM SET UP SO THAT THEY CAN WORK THROUGH THEIR SYSTEMS TO TRIGGER THIS OTA MECHANISM

WHICH IS PROBABLY THE MOST ACCELERATED PROCESS I'VE EVER SEEN OUT OF THE NIH AND THEIR ABILITY TO CONTRACT AND TO ACT AND TO MAKE DECISIONS.

SO THERE'S A WHOLE HOST OF ACTIVITIES THAT ARE CURRENTLY HAPPENING IN ORDER FOR THEM TO HAVE EVERYTHING READY TO GO ONCE THAT FIRST APPLICATION COMES IN.

THE UNDERSTANDING THAT WE HAVE AND WHAT I HAVE FROM THEM IS ALTHOUGH THEY NEED -- BECAUSE THEY'RE STILL THE NIH, THAT THEY'RE VERY CAUTIOUS ABOUT REALLY NAILING DOWN NUMBERS, BUT THEIR ASSURANCE IS THAT ONCE IT'S READY TO GO, THEY'LL HAVE THE MATCHING FUNDS READY TO GO BECAUSE GIVEN THEIR TOTAL BUDGET AND THE PRIORITY OF THIS PARTICULAR INITIATIVE WITHIN THE NHLBI SUPPORT FROM GARY GIBBONS AND FRANCES COLLINS, THAT THEY DON'T SEE THAT AS AN ISSUE IN TERMS OF THEIR FUNDS.

DR. JUELSGAARD: LET ME ASK THE QUESTION A LITTLE BIT DIFFERENTLY THEN. ASSUME A YEAR FROM NOW, FOR WHATEVER REASON, WE DECIDE WE'D RATHER ONLY SPEND $25 MILLION ON THIS INITIATIVE AND NOT 30. WILL WE BE FREE TO DO THAT?

DR. MILLAN: YES.

DR. JUELSGAARD: THANK YOU. THE THIRD THING, I THINK THIS IS IMPLIED IN WHAT WE'VE ALREADY

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GONE THROUGH, BUT YOU TALKED ABOUT SICKLE CELL PROJECTS. BUT JUST TO BE CLEAR, ANY PROJECT THAT CIRM WOULD FUND WOULD NEED TO BE A GWG MINIMUM SCORE OF 85, RIGHT?

DR. MILLAN: YES.

MR. THOMPSON: BUT THE CLINICAL PROGRAM HAS TIER I, II, III. SO, YES, THE APPLICATION WOULD HAVE TO RECEIVE A TIER I TO MOVE FORWARD.

DR. JUELSGAARD: GREAT. THANK YOU. THOSE ARE MY QUESTIONS.

MR. SHEEHY: OTHER QUESTIONS FROM OTHER BOARD MEMBERS? OKAY. SO SHALL WE GO TO A MORE GENERAL BUDGET DISCUSSION?

DR. LUBIN: CAN I MAKE ONE COMMENT. THIS IS A MAJOR ADVANCE FOR SICKLE CELL ANEMIA IN THE STATE OF CALIFORNIA. ONE OF THE PERSONS IN THE GOVERNMENT THAT'S BEEN A VERY STAUNCH ADVOCATE FOR SICKLE CELL IS BARBARA LEE. AND I'M SURE THAT -- I'M NOT SURE, BUT I'M WONDERING HOW MUCH SHE KNOWS THAT WE'RE GOING TO BE DOING THIS BECAUSE IT'S A PHENOMENAL OPPORTUNITY. I WOULD BE SURE TO PURSUE THAT. THAT'S NOT RELATED TO THE BUDGET OR ANY OF THE DETAILS OF THE MOU, BUT IT REALLY IS RELATED TO HER COMMITMENT TO SICKLE CELL, NOT THAT SHE COULD ADD ANY MONEY ONTO IT, BUT JUST TO KNOW THAT WE'RE

DOING THIS IS GREAT.

DR. MILLAN: THANK YOU SO MUCH.

MR. TORRES: MY INTENT WAS, ONCE WE'VE COMPLETED ACTION, TO GIVE BARBARA A CALL AND GIVE HER A BRIEFING ON THIS.

DR. LUBIN: I WOULD ABSOLUTELY DO THAT, AND I WOULD INVITE HER TO COME OVER TO CIRM AND DO A LITTLE PRESENTATION LIKE MARIA DID. I THINK IT WOULD BE GREAT.

MR. TORRES: WE HAVE AN INVITE OUT TO BARBARA, BUT I ALSO WANT TO MAKE SURE THAT I CONNECT WITH HER AS SOON AS WE'VE REACHED A DECISION HERE SO THAT SHE KNOWS.

DR. LUBIN: SOUNDS GOOD, AND I'D BE HAPPY TO HELP WITH THAT.

MR. TORRES: THANK YOU, BERT.

DR. JUELSGAARD: JUST TO BE CLEAR, WHAT WE WOULD DECIDE TODAY WOULD BE TO RECOMMEND A PLAN OF ACTION TO THE ICOC, RIGHT? WE'RE NOT -- WE DON'T HAVE THE AUTHORITY TO COMMIT TODAY $30 MILLION TO THIS PROGRAM; IS THAT RIGHT?

MR. SHEEHY: THAT'S RIGHT. WE'RE JUST MAKING A RECOMMENDATION TODAY.

DR. JUELSGAARD: GREAT. THANKS.

MR. SHEEHY: IS THERE ADDITIONAL

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DISCUSSION? WE DO HAVE THE BUDGET. I DON'T KNOW IF WE'RE STILL TALKING ABOUT THE MOU OR NOT, BUT I WAS HOPING WE COULD THEN ROLL THIS INTO A DISCUSSION ABOUT THE ALLOCATIONS FOR THE BUDGET AND SEE IF THERE ARE QUESTIONS OR COMMENTS OR CHANGES PEOPLE WANT TO MAKE IN THE BUDGET. IF YOU WANT TO GO BACK TO THAT.

DR. MILLAN: IT'S SLIDE 11. I'LL JUST SUMMARIZE AGAIN. SO FOR THE ALLOCATION, THE TOTAL ALLOCATION FOR THE CLINICAL PROGRAM WOULD BE 123 MILLION WITH 93 MILLION EARMARKED FOR THE GENERAL CLIN1/2 BUDGET, AND $30 MILLION SET ASIDE FOR CLIN1/2 PROGRAMS FOR THE SICKLE CELL CURE INITIATIVE, $20 MILLION FOR THE TRANSLATION PROGRAMS, AND $600,000 FOR EDUCATION PROGRAMS.

MR. SHEEHY: SO SINCE THERE'S NOT A DISCUSSION, I WOULD ENTERTAIN A MOTION TO RECOMMEND THE BUDGET TO THE FULL ICOC.

DR. DEAS: I MOVE THAT WE RECOMMEND THE BUDGET FORWARD.

MR. SHEEHY: SECOND?

CHAIRMAN THOMAS: SECOND.

MR. SHEEHY: PUBLIC COMMENT? I KNOW WE HAVE SOMEONE HERE IN SAN FRANCISCO, BUT IF ANY OF THE SITES, ANYONE FROM THE PUBLIC WOULD LIKE TO

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COMMENT.

DR. JUELSGAARD: I HAVE A COMMENT. THIS IS STEVE JUELSGAARD.

MR. SHEEHY: GO AHEAD, STEVE.

DR. JUELSGAARD: SO FOR THOSE PEOPLE THAT WEREN'T ON THE APPLICATION REVIEW SUBCOMMITTEE CALL EARLIER THIS MORNING, IF YOU RECALL BACK IN JULY WE APPROVED A NUMBER OF DISCOVERY PROGRAMS, BUT WE COULDN'T APPROVE ALL OF THEM THAT RANKED 85 AND ABOVE. WE WENT INTO A LARGE PROGRAMMATIC DISCUSSION AND APPROVED THOSE THAT WERE MOVED TO BE APPROVED, WHICH LEFT A NUMBER OF WHAT ARE VERY WORTHWHILE PROJECTS BOTH FROM THE SCIENTIFIC POINT OF VIEW, BUT ALSO FROM A THERAPEUTIC NEED POINT OF VIEW UNFUNDED. AND SO THOSE ARE STILL SITTING THERE, AND THIS PARTICULAR BUDGET PROPOSAL INCLUDES NO MONEY FOR DISCOVERY. YOU WILL SEE IT'S ZERO MILLION ON SLIDE 11.

I DID A LITTLE QUICK MATH ON THE AMOUNT OF MONEY THAT'S BEING ASKED FOR BY THE UNFUNDED PROJECTS, AND IT COMES TO JUST A LITTLE UNDER $8 MILLION. IT'S ABOUT $7.93 MILLION. AND WE HEARD FROM SOME OF THOSE PROGRAMS THIS MORNING DURING OUR APPLICATION REVIEW SUBCOMMITTEE MEETING, PEOPLE BOTH WERE INVESTIGATORS AS WELL AS PATIENTS, ABOUT THE

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NEEDS AND THE PROGRAMS THAT THEY HAD PUT FORTH TO THE GWG AND GOTTEN SCORES IN TIER I.

SO COUPLE THAT WITH THE FOLLOWING, THAT THE PRESENTATION THAT MARIA MADE ALLUDED TO THE FACT THAT THERE'S LIKELY TO BE SOME MONEY COMING BACK IN AGAIN THIS NEXT YEAR BECAUSE WE HAVE A HISTORY OF GETTING FUNDS BACK AGAIN. ALL OF THEM GET SPENT IN THE PROGRAMS THAT WE FUND FOR A VARIETY OF REASONS. IN HER PRESENTATION, SHE LEFT OUT THAT PARTICULAR PIECE AS PART OF THE BUDGETING ITEM, AND IT WOULD BASICALLY FLOW INTO THE FOLLOWING YEAR IN SOME FASHION. AND THAT MEANS IT WOULD GO INTO 2020, AND WE WOULD HAVE TO HAVE ADDITIONAL MONEY BEYOND THAT.

LET'S ASSUME FOR THE SAKE OF ARGUMENT IT'S $20 MILLION THAT COMES BACK. THAT'S A LOW NUMBER BASED ON HISTORICAL NORMS. ANYWAY, IF THAT MONEY COMES BACK AND WE DIDN'T HAVE ANY ADDITIONAL MONEY TO GO WITH IT, WE'D BE LEFT WITH A FAIRLY SMALL SUM OF $20 MILLION TO FIGURE OUT WHAT TO DO WITH.

AND I'M HOPEFUL THAT WE DO GET MONEY, BUT WE DON'T KNOW THAT WE WILL. SO THERE'S A PART OF ME THAT WANTS TO USE THAT UNSPENT, BUT LIKELY TO COME BACK MONEY TO HELP SUPPORT THE PROGRAMS THAT WE THOUGHT HIGHLY OF AT THIS LAST JULY MEETING BUT WE WERE UNABLE TO PROVIDE THE FUNDING FOR BECAUSE WE

DIDN'T HAVE THE BUDGET FOR IT.

MR. SHEEHY: I HAD A SUGGESTION FOR YOU, BUT GO AHEAD.

DR. JUELSGAARD: I'M DONE TALKING. I WAS MOVED THIS MORNING, JEFF, TO BE QUITE HONEST, BY THAT DISCUSSION OF ALL THE PEOPLE THAT WERE ON THE PHONE AND THE PLEAS THAT THEY WERE MAKING. WE HAVE SOME VERY WORTHWHILE PROGRAMS WE JUST SIMPLY LEFT BY THE SIDE OF THE HIGHWAY ON THIS ONE.

MR. SHEEHY: I FEEL THE SAME WAY. SO I WONDER -- WHAT I WAS GOING TO SUGGEST IS PERHAPS A FRIENDLY AMENDMENT TO THE BUDGET MOTION TO ALLOCATE 7.9 MILLION, THE FIRST 7.9 MILLION OF RETURNED FUNDS TO THOSE PROJECTS. AND MAYBE TO BE MORE CLEAR, WE WOULD FUND THEM SEQUENTIALLY IN ORDER OF REVIEW SCORE. RIGHT? BUT THERE'S SOME PATHWAY. I THINK, WHAT, THERE'S SEVEN LEFT, I THINK. THERE'S SIX. IF WE DON'T PROVIDE SOME CLARIFICATION, THEY'LL ONLY BE FUNDED IN ORDER OF -- WOULD ONLY GET FUNDED ONCE WE GOT THE FULL 7.9. IT'S NOT UNLIKELY THAT IT WILL DRIBBLE BACK IN COUPLE MILLION HERE, COUPLE MILLION THERE, BUT THERE'S AT LEAST SOME PROCESS.

IF YOU WANTED TO MAKE THAT FRIENDLY AMENDMENT, I WOULD SECOND IT. THAT KIND OF ADDRESSES. AND IF THE BRIDGE FUNDING DOES COME

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THROUGH, WE'VE ALREADY PRIORITIZED THIS FOR BRIDGE FUNDING. BUT IF IT DOESN'T, THEN THERE'S A WAY FORWARD FOR THOSE PROJECTS. BUT IT'S UP TO YOU.

DR. JUELSGAARD: I UNDERSTAND I RAISED THE ISSUE. I GUESS THERE ARE TWO WAYS OF DOING IT. ONE IS YOURS, WHICH REALLY LINES UP WITH THAT I WAS TALKING ABOUT. THE OTHER IS ESSENTIALLY TO SET ASIDE OUT OF THE BUDGET, WHETHER IT'S THE TRAN BUDGET OR THE CLIN BUDGET OR SOME OF BOTH, THAT $7.9 MILLION WITH THE MAKEUP TO TRAN AND CLIN, BUT WITH THE AMOUNT OF MONEY WE MIGHT HAVE TAKEN AWAY, THAT WOULD BE MADE UP WITH MONEY THAT COMES BACK IN. SO THAT WE GET THOSE PROJECTS UP AND RUNNING NOW AS OPPOSED TO ONE BY ONE OVER A PERIOD OF TIME.

AND SO I WAS HOPING THAT WE MIGHT BE WILLING TO DO THAT AND JUST GET THESE PEOPLE ON THEIR WAY WITH THEIR GREAT PROGRAMS. I'M SURE WE'LL BE ABLE TO MAKE UP THE DIFFERENCE WHEN MONEY COMES BACK IN FOR THIS BUDGET THAT'S ON PAGE 11. DR. MILLAN, WHAT DO YOU THINK?

MR. SHEEHY: MAYBE JUST SO WE HAVE A SENSE OF HOW IT MIGHT ALIGN WITH THE PLANNING BECAUSE THAT MAKES A LOT OF SENSE AS WELL. I'M ASSUMING THAT THE TRAN IS ONE OR TWO REVIEWS? SO IT'S ONE REVIEW. SO IT SEEMS LIKE THIS IS PROBABLY THE MOST LOGICAL

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PLACE TO TAKE IS CLIN BECAUSE THAT WON'T INTERFERE WITH -- THE TRAN, YOU'RE GOING TO HAVE TO SCHEDULE YOUR REVIEW. WE START IMPACTING THAT BUDGET NOW. WE MIGHT MAKE IT HARDER FOR THAT REVIEW. AND GIVEN THAT IT'S LIKELY ONLY TO BE ONE, PERHAPS LOGISTICALLY IT MAKES MORE SENSE TO TAKE IT OUT OF CLIN KNOWING THAT THE LAST REVIEW FOR CLIN OR THE LAST TWO REVIEWS ARE THE END OF 2019. BY THAT TIME SOME MONEY SHOULD HAVE COME BACK IN.

MR. TORRES: MR. CHAIRMAN, I AGREE WITH THE DISCUSSION AND DIRECTION THAT IT'S GOING AND IN SUPPORT OF WHAT STEVE AND YOU WANT TO DO. MY ONLY QUESTION IS UNDER THE INITIATIVE IT SAID THAT WE NEED TO GIVE PRIORITY TO EMBRYONIC STEM CELL PROJECTS, ESPECIALLY THOSE THAT MAY NOT RECEIVE FEDERAL FUNDING. OF THE DISCOVERY PROJECTS THAT WE HAVE, I'M FAMILIAR WITH MOST OF THEM, BUT ARE THEY ALL EMBRYONIC STEM CELL PROJECTS?

MR. SHEEHY: ONE OF THEM IS. I THINK TWO OF THEM.

MR. TORRES: I WOULD PREFER A PROPOSAL THAT, I GUESS, BOTH OF YOU SAID; THAT IS, IF WE CAN GET THEM ALL UNDER ONE UMBRELLA AND START TO FUND THEM ALL, THAT WOULD BE PREFERABLE. THE OTHER, OF COURSE, IF YOU HAVE TO DO THEM AD SERIATIM, WHAT

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KIND OF PRIORITY WOULD YOU INITIATE OTHER THAN JUST THE SCORES?

MR. SHEEHY: WHAT I'M HEARING IS ESSENTIALLY WE WOULD LIKE TO GO AHEAD AND GET THE WORK STARTED. IT DOES KIND OF MAKE SENSE TO TAKE EIGHT MILLION, I THINK IT'S CLOSER TO EIGHT, 7.9 AND SOME CHANGE, BUT THE EXACT NUMBER IS $7,929,593. SO MAYBE IF THAT'S THE MOTION YOU'D LIKE TO MAKE, STEVE, A MOTION TO MOVE THAT NUMBER OUT OF CLIN AND THEN POTENTIALLY HAVE THAT REPLACE -- THAT'S THEIR OTHER FUND.

MR. TORRES: I WOULD SECOND THAT MOTION IF THAT'S THE MOTION BEING PUT FORTH.

MR. SHEEHY: FROM THE ACTUAL BUDGET.

MR. TOCHER: FROM RECOVERED FUNDS, IF I UNDERSTAND. I UNDERSTAND, BUT THE CLIN --

MR. SHEEHY: BACKFILL TO OTHER FUNDS. SO PART OF THE MOTION WOULD ALSO BE TO BACKFILL THE CLIN CUT FROM RECOVERED FUNDS.

MR. TOCHER: THAT'S RIGHT. AND WE HAVE A PENDING MOTION FROM DR. DEAS AND DR. THOMAS. WITH THEIR PERMISSION AS A FRIENDLY AMENDMENT. DR. DEAS?

DR. DEAS: I'M HERE, AND THAT'S FINE WITH ME.

MR. SHEEHY: CHAIRMAN THOMAS?

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CHAIRMAN THOMAS: AGREED.

DR. MILLAN: JUST RESPONDING. THANK YOU FOR THE OPPORTUNITY TO SPEAK ON THIS. I THINK THAT THAT'S A REASONABLE APPROACH. AND I THINK THAT IN THAT CASE WHAT I WOULD PROPOSE THEREFOR, JUST TO SIMPLIFY MATTERS, INSTEAD OF OUR PLAN TO CARRY OVER ANY OF THE RETURNED FUNDS FOR 2020, TO JUST BRING IT INTO THE BUDGET FOR THIS COMING YEAR, 2019, FOR THE WHOLE BUDGET.

AND THEN I WOULD THEN PROPOSE THAT WHATEVER RESIDUAL FUNDS COME BACK, SO WE WOULD TAKE WHATEVER IS REQUIRED TO FUND THOSE DISCOVERY PROGRAMS THAT WE SPOKE OF, WE TAKE OUT OF THE CLIN BUDGET, WE BACKFILL THE CLIN BUDGET, BUT THEN ANYTHING BEYOND THAT WE WOULD LIKE TO GO AHEAD AND HAVE IT IN 2019, AND I'D LEAVE IT UP TO THE BOARD AS TO WHETHER YOU WANT US TO COME BACK TO YOU ONCE WE HAVE THE REST OF THE 30 MILLION, THE ESTIMATED 30 MILLION, TO ALLOCATE IT OR CAN WE PUT IT TOWARDS CLIN. WE'RE ALREADY GOING TO BE SHORT ON WHAT WE PLANNED FOR FOR THE CLINICAL STAGE PROGRAMS.

MR. SHEEHY: I DON'T KNOW WHAT OTHER PEOPLE'S THOUGHTS ARE. I MEAN I PERSONALLY WOULD LIKE TO SEE WHERE WE ARE WHEN THE FUNDS START COMING BACK IN. WE MAY NOT -- WE UNDERSPENT ON CLIN THIS

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YEAR. AND THE OTHER THING IS, DEPENDING ON HOW RAPIDLY THE MOU AND THE SICKLE CELL PROJECTS SCALE, THERE MAY BE SOMEPLACE ELSE WHERE WE MIGHT WANT TO CONSIDER. SO I JUST WOULDN'T WANT TO TIE DOWN THOSE FUNDS OTHER THAN BACKFILLING THE EIGHT MILLION.

DR. MILLAN: THAT'S FINE.

MR. SHEEHY: THAT'S MY VIEW.

DR. MILLAN: WE CAN ALWAYS BRING IT BACK TO THIS BOARD MIDYEAR OR SOMETHING IF IT TURNS OUT THAT WE HAVE THE RECOVERED FUNDS AND WE PROPOSE TO DO -- THAT WE'D LIKE TO ALLOCATE IT TO ONE PROGRAM OR ANOTHER.

MR. SHEEHY: SCOTT TOCHER, KIND OF GIVE US WHERE WE ARE ON THE MOTION, AND THEN WE CAN HAVE ANOTHER ROUND OF COMMENTS SO THAT WE'RE CLEAR BECAUSE WE DO HAVE A COUPLE OF DIFFERENT MOVING PARTS HERE. AND THEN ONCE WE DO THAT, WE'LL COME BACK TO PUBLIC COMMENT.

MR. TOCHER: SURE. THANKS, JEFF. SO AS I HAVE IT, THE MOTION MADE BY DR. DEAS AND SECONDED BY J.T. WAS AMENDED BY FRIENDLY AMENDMENT TO ACCEPT THE BUDGET AS PROPOSED EXCEPT TO REMOVE THE REQUIRED $7.9 PLUS MILLION DOLLARS FROM THE NON-SCD BUDGET OF 93 MILLION AND TO USE THOSE FUNDS IMMEDIATELY TO FUND THE UNFUNDED QUEST APPLICATIONS THAT WERE

CONSIDERED AND ARE BEING POSTPONED TO A FUTURE MEETING FROM EARLIER THIS MORNING TO BACKFILL DURING 2019 THIS 7.9 FROM THE CLIN1 AND 2 BUDGET WHICH RECOVERED FUNDS AS THEY COME IN. THAT ONCE THAT IS REPLENISHED, WE WILL COME BACK TO THE BOARD FOR FURTHER DETERMINATION ON WHAT IT WOULD LIKE TO DO WITH ANY FURTHER RECOVERED FUNDS.

MR. SHEEHY: DOES THAT CAPTURE EVERYTHING? FIRST, DO THE MAKERS ON BOTH OF THE MOTION, WHICH WOULD BE DR. DEAS AND CHAIRMAN THOMAS, AND THEN MR. JUELSGAARD WHO MADE THE FRIENDLY AMENDMENT? AND I THINK WAS THERE A SECOND.

MR. TOCHER: THERE WAS. SENATOR TORRES.

MS. BONNEVILLE: AND YOU SAID YOU WOULD.

MR. SHEEHY: I'M OKAY WITH IT. I JUST WANT TO MAKE SURE EVERYBODY ELSE IS OKAY WITH THAT.

DR. DEAS: YES, I AM.

MR. TORRES: ME TOO.

MR. JUELSGAARD: YES, I'M VERY MUCH ON BOARD WITH THIS MODIFIED MOTION.

MR. SHEEHY: ANY DISCUSSION ABOUT THE MOTION FROM BOARD MEMBERS BEFORE I OPEN UP TO PUBLIC COMMENT? OKAY. SO WE HAVE DON REED HERE.

MR. REED: I WANT TO MAKE SURE I UNDERSTAND IT. DO I UNDERSTAND CORRECTLY THAT SOME

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OF THE PEOPLE WHO HAVE MADE PROPOSALS THIS MORNING ARE GOING TO BE VERY HAPPY?

MR. SHEEHY: DEPENDS ON WHAT ACTION THE BOARD TAKES NEXT WEEK. IF THIS MOTION PASSES, IT WILL BE RECOMMENDED THAT THEY RECEIVE THE FUNDING.

MR. REED: I HOPE THAT YOU LET THEM KNOW RIGHT AWAY. THIS IS WONDERFUL. THANK YOU SO MUCH.

MR. SHEEHY: ADDITIONAL PUBLIC COMMENT AT ANY SITES?

OPERATOR: LADIES AND GENTLEMEN, IF YOU WANT TO ASK A QUESTION, PLEASE PRESS STAR AND THEN ONE.

MR. SHEEHY: HEARING NONE, PUBLIC COMMENT IS CLOSED. AND WE CAN --

MR. TOCHER: FOR THE ROLL CALL THAT MARIA WILL BE CALLING, MEMBERS MELMED AND VUORI WILL NOT BE CALLED BECAUSE THEY HAVE INTEREST IN APPLICATIONS THAT ARE CURRENTLY PENDING IN THE QUEST ROUND.

MR. SHEEHY: THANK YOU.

MS. BONNEVILLE: DEBORAH DEAS.

DR. DEAS: YES.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: BERT LUBIN.
DR. LUBIN: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: THANK YOU.
MR. SHEEHY: MOTION CARRIES. SO NOW I BELIEVE WE HAVE DR. SAMBRANO WITH SOME CHANGES IN THE CONCEPT PLANS.
DR. SAMBRANO: THANK YOU, MR. SHEEHY. YES, THAT'S CORRECT. SO ON THE SLIDE I'M GOING TO PRESENT SOME OF THE PROPOSED CONCEPT CHANGES THAT WOULD COVER WHAT WE TALKED ABOUT REGARDING THE NHLBI/CIRM CURE SICKLE CELL DISEASE JOINT INITIATIVE AS WELL AS OTHERS THAT AFFECT THE TRANSLATIONAL AND CLINICAL PROGRAMS. AND THESE LARGELY TEND TO REMOVE SMALL MOLECULES AND BIOLOGICS FROM ELIGIBILITY FOR NEW PROJECTS COMING INTO CIRM AS OPPOSED TO PIPELINE PROJECTS, BY WHICH WE MEAN THOSE WHERE WE HAVE

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PREVIOUSLY FUNDED THE CANDIDATE THAT THEY ARE DEVELOPING THROUGH CIRM FUNDS. SO I'LL EXPLAIN THAT IN MORE DETAIL THROUGH THESE SLIDES.

FIRST, I'M GOING TO ADDRESS THE TRANSLATIONAL PROGRAM. AND THIS FIRST SLIDE ON THE TRANSLATIONAL PROGRAM DEPICTS WHAT THE PROGRAM LOOKS LIKE TODAY. IT IS A PROGRAM THAT ACCEPTS APPLICATIONS FROM THOSE WHO HAVE CONDUCTED PROOF OF CONCEPT STUDIES, AND THOSE CAN COME FROM THINGS SUCH AS THE DISC2 PROGRAM, WHICH PRODUCES CANDIDATES WITH CONCEPTS THAT ARE READY FOR TRANSLATION. AND SO THAT FEEDS INTO THE TRANSLATION PROGRAM AND WILL SUPPORT DIFFERENT PRODUCT TYPES, INCLUDING A THERAPEUTIC, DEVICES SUCH AS A DIAGNOSTIC OR OTHER MEDICAL DEVICE, AND ALSO TOOLS. EACH OF THESE DIFFERENT PRODUCT TYPES HAVE DIFFERENT TIMELINES AND MAXIMUM BUDGETS TO ADDRESS THE ACTIVITIES THAT THEY WOULD CONDUCT UNDER THE TRANSLATIONAL PROGRAM. THE THERAPEUTIC IS REALLY THE LARGEST. I THINK 95 PERCENT OR MORE OF THE APPLICATIONS WE GET ARE FOR THE TRAN1 OR THE THERAPEUTIC TYPE. AND THAT INCLUDES SMALL MOLECULES, BIOLOGICS, AND CELL THERAPIES.

WHAT WE ARE PROPOSING IN TERMS OF CHANGING, PART OF THIS IS IN THE CONCEPT, PART OF IT

IS OUR INTENT IN TERMS OF WHAT WE WOULD SOLICIT IN TERMS OF APPLICATIONS. SO, FIRST, OUR INTENT FOR NEXT YEAR WOULD BE TO SOLICIT APPLICATIONS ONLY FOR THE TRAN1 OR THERAPEUTIC PROGRAM AND NOT FOR THE OTHER PRODUCT CANDIDATE TYPES. AND THEN FOR PIPELINE PROJECTS, SO THOSE THAT HAVE RECEIVED CIRM FUNDING BEFORE, THEY WOULD BE ELIGIBLE TO APPLY, WHETHER THEY'RE A SMALL MOLECULE, BIOLOGIC, OR CELL THERAPY, BUT FOR ANY NEW PROGRAMS THAT ARE COMING IN, WE WOULD ONLY ACCEPT APPLICATIONS THAT ARE PROPOSING A CELL THERAPEUTIC.

SO THAT WOULD BE THE PROPOSED CHANGE FOR THE TRANSLATION PROGRAM.

FOR THE CLINICAL PROGRAM, THIS IS WHAT THE CURRENT PROGRAM LOOKS LIKE FOR THE CLIN1 AND CLIN2, WHICH COVERS THE IND-ENABLING TYPE OF PROJECTS AS WELL AS THE CLINICAL TRIAL PROJECTS. SO, OF COURSE, THE TRANSLATION PROGRAM FEEDS INTO THE CLIN1 OR IND-ENABLING, AND THE OBVIOUS DIFFERENCE IS ONE BEING IND-ENABLING VERSUS A CLINICAL TRIAL. THE AMOUNT OF TIME IS DIFFERENT FOR EACH, 18 MONTHS FOR THE CLIN1 AND WE ALLOW UP TO FOUR YEARS FOR THE CLINICAL TRIAL. BUT BOTH GENERALLY HAVE ACCEPTED THERAPEUTIC CANDIDATES THAT INCLUDE SMALL MOLECULES, BIOLOGICS, CELL THERAPIES, AND DEVICES.

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SO OUR PROPOSAL IN TERMS OF WHAT WE WOULD CHANGE FOR THE CLINICAL PROGRAM WOULD BE THAT FOR THE CLIN2 OR CLINICAL TRIALS, THERE WOULD BE NO CHANGE. WE WOULD CONTINUE TO ACCEPT BOTH NEW AND PIPELINE PROJECTS AS WE CURRENTLY DO. BUT FOR THE CLIN1, THE IND-ENABLING, WE WOULD BASICALLY MIRROR WHAT WE'RE DOING FOR TRAN, WHICH IS FOR ANY PIPELINE PROJECTS, WE WOULD CONTINUE TO ACCEPT ALL DIFFERENT PRODUCT TYPES. NEW PROJECTS WE WOULD LIMIT TO CELL THERAPY ONLY.

IN ADDITION, WE WOULD ADD MESENCHYMAL STEM CELLS, OFTEN KNOWN AS MSC'S, TO A STATEMENT WITHIN THE CONCEPT OR PROGRAM ANNOUNCEMENT WHICH ACTUALLY CREATES A LITTLE BIT OF A HIGHER BAR FOR THESE TYPES OF APPLICATIONS TO COME IN. SO THAT IN THE STATE, AND I'LL READ IT TO YOU, IN THE ELIGIBILITY, WE HAVE MINIMALLY MANIPULATED BONE MARROW, MINIMALLY MANIPULATED CORD BLOOD, OR UNMODIFIED HEMATOPOIETIC STEM CELLS, AND THEN WE WOULD ADD THE MSC'S ARE ELIGIBLE ONLY IF BEING DEVELOPED AS A NOVEL METHOD OF ADDRESSING A RARE OR UNMET NEED UNLIKELY TO RECEIVE FUNDING FROM OTHER SOURCES. SO THE MSC'S HAVEN'T BEEN PREVIOUSLY HELD TO THAT BAR, BUT WOULD BE UNDER THIS PROPOSAL.

AND THEN FINALLY THERE WOULD BE CHANGES IN

THE CONCEPT TO ADDRESS WHAT WAS DISCUSSED RELATED TO THE CIRM/NHLBI SICKLE CELL DISEASE JOINT INITIATIVE. THOSE WOULD INCLUDE THE FACT THAT ALL SICKLE CELL DISEASE APPLICATIONS THAT WOULD COME TO US WOULD BE CONSIDERED UNDER THIS JOINT FUNDING MECHANISM, THAT APPLICATION MATERIALS WOULD BE SHARED WITH NHLBI, THAT NON-CALIFORNIA APPLICANTS MAY APPLY FOR NHLBI FUNDS TO COVER UNALLOWABLE ACTIVITIES OUTSIDE OF CALIFORNIA, AND, FINALLY, THAT CO-FUNDED PROJECTS MUST ADHERE TO POLICIES OF NHLBI AND NIH FOR DATA AND SAFETY MONITORING AND DATA SHARING, INCLUDING A DATA COORDINATING CENTER THAT IS IN THE PROCESS OF BEING SET UP SPECIFICALLY FOR THIS CURE SICKLE CELL DISEASE INITIATIVE.

SO THOSE ARE THE PROPOSED CHANGES. HAPPY TO TAKE QUESTIONS.

MR. SHEEHY: QUESTIONS FROM BOARD MEMBERS?

DR. VUORI: CAN I ASK A QUICK CLARIFICATION ABOUT GENE THERAPY? IF YOU DO IN VIVO GENE THERAPY, DO YOU CONSIDER THAT BIOLOGICS OR CELL THERAPY?

DR. SAMBRANO: SO THE SMALL MOLECULES AND BIOLOGICS, THE WAY THAT THE LANGUAGE CURRENTLY ALLOWS THE PROJECT TO COME IN IS THAT THERE MUST BE A STEM CELL INVOLVED IN SOME WAY. SO THE GENE

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THERAPY WOULD GENERALLY FALL, IF IT'S BY ITSELF AND DOES NOT INVOLVE A STEM CELL, SUCH AS A CELL THERAPY, SO YOU CAN DO A GENE-MODIFIED CELL THERAPY, OTHERWISE, IT WOULD BE A BIOLOGIC AND WOULD NOT QUALIFY.

DR. VUORI: OKAY. THANKS.

MR. SHEEHY: SMALL MOLECULE OR A BIOLOGIC, THEN IN VIVO MODIFIED STEM CELL, THAT WOULD STILL BE IN SCOPE?

DR. SAMBRANO: IT WOULD NOT. IT IS IN SCOPE CURRENTLY BECAUSE IT MUST INVOLVE A STEM CELL IN SOME WAY TO BE ELIGIBLE. BUT WHAT WE ARE PROPOSING IS THAT WE WOULD LIMIT THE PROJECTS ONLY TO THOSE THAT ARE PROPOSING A THERAPEUTIC THAT'S A CELL THERAPY.

MR. SHEEHY: SOME OF THE SCIENTISTS ON HERE MAY KNOW MORE THAN I KNOW. DO WE WANT TO CONTINUE TO LEAVE A DOOR FOR JUST GENE THERAPY IF IT'S TO DO IN VIVO GENE THERAPY TO MODIFY A STEM CELL? THAT IS NOT SOMETHING I PERSONALLY THOUGHT OF. I DON'T KNOW -- OBVIOUSLY THAT'S WHERE A LOT OF THIS IS GOING TO END UP, IN VIVO. AND I DON'T KNOW, THAT'S OPPORTUNITIES WE MIGHT MISS. HAVING GOTTEN THE HORSE TO THE WATER, YOU MIGHT WANT TO BE THERE WHEN IT TAKES A DRINK. I DON'T KNOW. DR. VUORI,

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YOU ASKED THAT QUESTION.

DR. VUORI: CERTAINLY. SO AS IT COMES TO GENE THERAPY, THERE ARE TWO WAYS OF MANIPULATING CELLS BY GENE THERAPY. ONE IS EX VIVO. SO YOU TAKE THE CELLS FROM THE BODY -- THEY CAN BE STEM CELLS -- MODIFY THEM BY GENE THERAPY AND PUT THEM BACK INTO THE PATIENT. SO YOU MIGHT BE CORRECTING A GENE DEFECT, FOR EXAMPLE.

THE OTHER APPROACH IS TO HAVE THE GENE THERAPY APPROACH IN VIVO WHERE YOU HAVE A MATURE VIRUS AND THAT DELIVERS A PAYLOAD OF CERTAIN CELLS. AND YOU MODIFY THE GENE OR GENOME OF A TARGET CELL DIRECTLY IN THE HUMAN BODY. AND I THINK IN SOME APPLICATIONS, ESPECIALLY IF IT COMES TO CENTRAL NERVOUS SYSTEM, THERE IS BRAIN DISORDERS. I THINK GREAT PROGRESS IS BEING MADE IN THIS LATTER, THIS IN VIVO GENE THERAPY, WHERE THERE ARE VARIOUS TYPES OF ESSENTIALLY AAV VIRUSES THAT CAN AFFECT BRAIN CELLS IN A DESIRED MANNER IN THE BODY. MANY TIMES THESE ARE STEM CELLS. CERTAINLY FROM MY PERSPECTIVE, FROM SCIENTIFIC PERSPECTIVE, IT WOULD BE UNFORTUNATE IF THAT REALLY RAPIDLY MOVING AND VERY PROMISING AREA WOULD NOT BE ELIGIBLE FOR CIRM FUNDING, BUT AT THE SAME TIME CIRM CANNOT FUND EVERYTHING. SO IT'S A MATTER OF DISCUSSION AS TO WHAT MAKES THE MOST

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SENSE.

MR. SHEEHY: I PERSONALLY WOULD BE SUPPORTIVE OF EXPANDING THE SCOPE IN THAT PARTICULAR AREA. I THINK THAT ALSO MAY BE RELEVANT TO THE SICKLE CELL PROJECT AS WELL. DR. MILLAN, DO YOU HAVE ANYTHING ON THAT?

DR. MILLAN: WE'RE SUPPORTIVE OF THAT. THERE'S BEEN GREAT PROGRESS, AND IT'S COMPATIBLE WITH THE ADVANCEMENTS AND GENOMICS TO INFORM TARGETS.

MY QUESTION IS ARE YOU PROPOSING TO MAKE IN SCOPE GENE THERAPY THAT SPECIFICALLY TARGETS STEM CELLS OR GENE THERAPY PERIOD? IT IS ALLOWABLE WITHIN SOME OF THE LANGUAGE IN PROP 71. WE JUST HAVEN'T BEEN INTERPRETING IT IN THAT WAY.

MR. SHEEHY: I SUSPECT STEM CELLS AT LEAST AT THIS POINT.

DR. MILLAN: OKAY.

DR. VUORI: I DON'T KNOW IF BERT AND SHLOMO WANT TO WEIGH IN ON THIS ONE.

DR. MELMED: I AGREE WITH YOU. I AGREE WITH YOUR APPROACH.

DR. LUBIN: THE APPROACH BEING SPECIFIC FOR STEM CELLS OR BROADER THAN THAT? I THINK BROADER IS WHERE WE ARE HEADED IN A VARIETY OF

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AREAS, AND THERE'S SOME REALLY EXCITING THINGS. SO I WOULDN'T WANT TO RESTRICT AN OPPORTUNITY THAT REALLY WOULD BE A GOOD THING FOR CIRM TO HAVE.

DR. MELMED: ABSOLUTELY. IT'S GOT TO BE AS BROAD AS POSSIBLE IN THE CONTEXT OF THE STEM CELLS. GENE THERAPY AND EXOSOMES WITHIN THE STEM CELL WOULD BE IDEAL THERAPEUTIC CANDIDATES.

MR. SHEEHY: I'M TRYING TO GET A SENSE OF WHERE WE ARE. DR. MILLAN OR DR. SAMBRANO.

DR. SAMBRANO: I THINK THAT'S A FINE PROPOSAL. I THINK WE NEED -- OUR LEGAL COUNSEL WASN'T HERE, BUT I THINK WE NEED TO UNDERSTAND THE EXTENT TO WHICH WE ARE ABLE TO FUND RESEARCH OUTSIDE OF STEM CELLS.

DR. MELMED: NOT OUTSIDE STEM CELLS.

DR. SAMBRANO: I THINK THE PROPOSAL WAS GENE THERAPY IN GENERAL. AND IF THERE IS NO STEM CELL, I DON'T KNOW THAT WE CAN LEGALLY FUND IT. BUT IF IT'S GENE THERAPY THAT TARGETS A STEM CELL, THEN ABSOLUTELY WE COULD.

DR. MELMED: KRISTINA, THAT'S WHAT YOU WERE SAYING, RIGHT, WITHIN STEM CELL CONTEXT?

DR. VUORI: RIGHT. YEAH. I THINK BERT THEN CHIMED IN AND EXPANDED IT A LITTLE BIT.

DR. MILLAN: SCOTT TOCHER IS IN THE ROOM.

SCOTT, WE'RE DISCUSSING ALLOWING GENE THERAPY FOR STEM CELLS. BUT THEN THE QUESTION AROSE IN TERMS OF GENE THERAPY AS ALLOWABLE WITHIN PROP 71. AND THE QUESTION IS IS IT ALLOWABLE UNDER PROP 71, VITAL RESEARCH OPPORTUNITY?

MR. TOCHER: RIGHT. SO THE PROPOSITION ALLOWS, UNDER CERTAIN CONDITIONS AS YOU DESCRIBE, THIS TYPE OF FUNDING BASED UPON A VOTE OF THE GRANTS WORKING GROUP THAT WOULD OCCUR THAT MAKES THAT FINDING AT A CERTAIN THRESHOLD. SO THAT WOULD BE SOMETHING THAT WOULD BE, ASSUMING THAT MEETS THE CRITERIA WITHIN THE PROPOSITION, WHICH I'M UNABLE TO PULL UP AT THE MOMENT.

DR. MILLAN: IT'S UNDER THE TITLE "RESEARCH OPPORTUNITY."

MR. SHEEHY: OKAY. SO DO WE HAVE CONSENSUS THAT WE SHOULD EXPAND IT AT A MINIMUM FOR IN VIVO GENE THERAPY FOR STEM CELLS?

DR. VUORI: YES.

MR. SHEEHY: AND THEN I GUESS WE'LL HEAR FROM COUNSEL WHETHER IN VIVO GENE THERAPY FOR ANY CELL TYPE IS ALLOWABLE, AND THEN WE CAN COME TO A CONSENSUS ONE WAY OR ANOTHER ON THAT.

DR. MILLAN: WOULD YOU LIKE US TO BRING IT TO THE FULL BOARD?

DR. JUELSGAARD: WHILE WE ARE WAITING FOR THAT, CAN I ASK A QUESTION ABOUT THE SICKLE CELL DISEASE PROGRAM?

MR. SHEEHY: PLEASE.

DR. JUELSGAARD: SO WE'RE PROPOSING BASICALLY A NARROWING OF THE NUMBER OF CANDIDATES THAT MIGHT COME IN GENERALLY FOR FUNDING AND ELIMINATING THOSE, EITHER THE SMALL MOLECULE OR BIOLOGICAL AREA, UNLESS WE'VE PREVIOUSLY FUNDED THEM SO THAT THERE'S SORT OF A CHAIN OF CIRM FUNDING INVOLVED. AND IN THOSE CASES WE WOULD CONSIDER THEM. DOES THAT APPLY TO THE SICKLE CELL PROGRAM AS WELL? THAT WAS MY READING OF HOW THIS WORKED, BUT I WANT TO BE SURE OF THAT.

DR. SAMBRANO: YES, THAT'S THE CURRENT INTERPRETATION.

MR. SHEEHY: DO WE WANT TO FOR THE SICKLE CELL INITIATIVE MAINTAIN THE PRIOR SCOPE SO WE CAN ABSORB AS MANY PROJECTS AS POSSIBLE?

DR. DEAS: I THINK WE SHOULD DO THAT, THE BROADEST SCOPE.

MR. SHEEHY: MY FEELING AS WELL. WE'RE OBVIOUSLY IN A RACE THAT WE HOPE WE WILL WIN AS SOON AS POSSIBLE. PERHAPS IN THAT CIRCUMSTANCE WE CAN DO A BROADER SCOPE.

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DR. MILLAN: YES, WE AGREE WITH THAT. SCOTT HAS SOMETHING ON THE GENE THERAPY.

MR. TOCHER: YES. I JUST WANTED TO BACK UP WHAT WE HAD SPOKEN OF A MOMENT AGO. THE PROPOSITION ALLOWS THAT THE AGENCY MAY FUND OTHER SCIENTIFIC AND MEDICAL RESEARCH AND TECHNOLOGIES IN STEM CELL RESEARCH THAT RECEIVE AT LEAST A TWO-THIRDS RECOMMENDATION BY THE GRANTS WORKING GROUP THAT SUCH A RESEARCH PROPOSAL IS A VITAL RESEARCH OPPORTUNITY. THE ABILITY IS THERE.

MR. SHEEHY: AMONGST THE BOARD MEMBERS -- FIRST, FROM THE TEAM, WHAT'S YOUR FEELING BEYOND STEM CELLS?

DR. MILLAN: FOR GENE THERAPY FOR VITAL RESEARCH OPPORTUNITIES TO ADDRESS UNMET MEDICAL NEEDS, WE WOULD BE IN SUPPORT OF THAT.

MR. SHEEHY: WHAT ABOUT MY COLLEAGUES? WHAT'S THE SENSE? BROADER?

CHAIRMAN THOMAS: I WOULD AGREE THAT BROADER IS BETTER HERE. WE JUST DON'T WANT TO PRECLUDE THE OPPORTUNITY IF SOMETHING REALLY GOOD COMES ALONG THAT'S ON POINT. SO I WOULD AGREE WITH THE BROADER APPROACH.

MR. TORRES: MY ONLY CONCERN IS THAT WE ARE VERY CAREFUL -- I THINK SCOTT ALREADY SAID SO --

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THAT WE COMPLY WITHIN THE PROPOSITION'S INTENT.

CHAIRMAN THOMAS: ABSOLUTELY.

MR. SHEEHY: SO I ASSUME THAT WE CAN WORK THAT OUT IN THE GWG PROCESS WHERE WE FORMALLY ACKNOWLEDGE IT'S A VITAL RESEARCH OPPORTUNITY.

MR. TOCHER: THAT'S RIGHT.

MR. SHEEHY: PERHAPS WHEN THEY COME IN, WE CAN GET THAT APPROVED BEFORE WE ACTUALLY REVIEW IT. MAYBE THAT'S THE PROCESS. MARIA IS NODDING.

SO ARE THERE OTHER COMMENTS OR QUESTIONS ABOUT THE SCOPE? I WAS GOING TO TRY TO PUT THIS INTO A MOTION.

SO I THINK OUR MOTION WOULD BE TO APPROVE ALLOWING -- HAVING WITHIN SCOPE IN VIVO GENE THERAPY OF STEM CELLS. AND THEN IF IT FITS A VITAL RESEARCH OPPORTUNITY, IN VIVO MODIFICATION -- IN VIVO GENE THERAPY FOR OTHER CELL TYPES. AND THEN THE THIRD IS TO RETAIN THE ORIGINAL SCOPE OF PROJECTS THAT COME IN WITHIN THE SICKLE CELL INITIATIVE FOR CLIN1 AND CLIN2.

SO DOES THAT KIND OF CAPTURE EVERYTHING THAT WE'VE SAID TODAY?

MS. BONNEVILLE: CLIN1, THE ORIGINAL SCOPE OR THE SCOPE THAT WAS --

MR. SHEEHY: WILL BE MAINTAINED FOR THE

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SICKLE CELL INITIATIVE.

MR. TOCHER: CHANGES PROPOSED WOULD NOT BE --

MR. SHEEHY: EXCEPT FOR THE SICKLE CELL --

DR. MILLAN: SO THE SICKLE CELL INITIATIVE PROGRAMS WOULD BE EXEMPT FROM THE NEW CLIN1 RESTRICTIONS.

MR. SHEEHY: ANY OTHER THOUGHTS, CHANGES, ETC.? I THINK THIS IS GREAT. SO IS IT POSSIBLE TO RESTATE?

CHAIRMAN THOMAS: WHEN YOU'RE RESTATING, MR. TOCHER, INDICATE WHETHER THIS IS SHORTER OR LONGER THAN THE MOTION WE HAD EARLIER.

MR. TOCHER: THANK YOU FOR THE OPPORTUNITY. LET ME STATE AT LEAST TWO COMPONENTS OF IT BECAUSE I BELIEVE THAT THERE MAY BE A THIRD THAT I MAY NOT HAVE.

SO THE MOTION IS TO RECOMMEND THAT THE BOARD APPROVE THE PROPOSED CONCEPT CHANGES EXCEPT AS FOLLOWS: THAT THE CONCEPT PLAN FOR CLIN1 BE CHANGED TO ALLOW IN VIVO GENE THERAPY OF STEM CELLS. SECONDLY, THAT THE SICKLE CELL DISEASE INITIATIVE BE EXEMPT FROM THE CLIN1 PROPOSED NEW RESTRICTIONS.

AND I KNOW THERE'S A THIRD.

DR. MILLAN: GENE THERAPY -- VITAL

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RESEARCH OPPORTUNITY FOR OTHER GENE THERAPY NOT TARGETING STEM CELLS. THAT GENE THERAPY, IN GENERAL, PROJECTS COULD BE BROUGHT TO THE GWG FOR CONSIDERATION WITH A TWO-THIRDS VOTE IN TERMS OF WHETHER IT'S A VITAL RESEARCH OPPORTUNITY THAT SHOULD BE FULLY REVIEWED BY THE GWG.

MR. TOCHER: A RESTATEMENT OF THE PROPOSITION'S PROVISION THAT VITAL RESEARCH OPPORTUNITIES AS DETERMINED BY THE GRANTS WORKING GROUP WITH THE TWO-THIRDS MAJORITY.

DR. MILLAN: FOR GENE THERAPY THAT DON'T SPECIFICALLY TARGET STEM CELLS.

MR. SHEEHY: DO WE WANT TO INCLUDE THE GENE THERAPY CHANGES FOR THE TRAN PROGRAM AS WELL? YES.

SO ONE FURTHER MODIFICATION IS TO INCLUDE THE GENE THERAPY CHANGES FOR THE TRAN PROGRAM AS WELL. I THINK WE KIND OF --

MR. TOCHER: WHO'S THE MAKER?

MR. SHEEHY: I'LL MAKE THE MOTION. IS THERE A SECOND?

CHAIRMAN THOMAS: SECOND.

MR. SHEEHY: SO IS THAT CLEAR TO EVERYONE? WE HAVE THUMBS UP FROM THE TEAM. DO WE HAVE ANY PUBLIC COMMENT? NO PUBLIC COMMENT. HEARING NO

PUBLIC COMMENT AT ANY OF THE SITES, CAN WE GO TO A VOTE THEN PLEASE.

MS. BONNEVILLE: DEBORAH DEAS.

DR. DEAS: YES.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: BERT LUBIN.

DR. LUBIN: YES.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: YES.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: KRISTINA VUORI.

DR. VUORI: YES.

MS. BONNEVILLE: THANK YOU. MOTION CARRIES.

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MR. SHEEHY: OKAY. DO WE HAVE ANY GENERAL PUBLIC COMMENT AT ANY OF THE SITES? WITH THAT, THE MEETING IS ADJOURNED. THANK YOU, EVERYONE, FOR TAKING YOUR TIME THIS AFTERNOON.

(THE MEETING WAS ADJOURNED AT 3:19 PM.)

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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 TELEPHONIC PROCEEDINGS BEFORE THE SCIENCE SUBCOMMITTEE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE IN THE MATTER OF ITS REGULAR MEETING HELD 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.

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