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: VIA ZOOM

DATE: MARCH 7, 2022
1 P.M.

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

FILE NO.: 2022-10

I N D E X

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MONDAY, MARCH 7, 2022; 1 P.M.

CHAIRMAN GOLDSTEIN: OKAY. LET ME CALL US TO ORDER FOR TODAY'S SCIENCE SUBCOMMITTEE, AND THEN ASK MARIA BONNEVILLE TO CALL THE ROLL.

MS. BONNEVILLE: HAIFA ABDULHAQ. DEBORAH DEAS.

DR. DEAS: HERE.

MS. BONNEVILLE: MARK FISCHER-COLBRIE.

DR. FISCHER-COLBRIE: HERE.

MS. BONNEVILLE: ELENA FLOWERS. JUDY GASSON.

DR. GASSON: HERE.

MS. BONNEVILLE: LARRY GOLDSTEIN.

CHAIRMAN GOLDSTEIN: HERE.

MS. BONNEVILLE: DAVID HIGGINS. PAT LEVITT.

DR. LEVITT: HERE.

MS. BONNEVILLE: DAVE MARTIN. SHLOMO MELMED.

DR. MELMED: HERE.

MS. BONNEVILLE: CHRISTINE MIASKOWSKI.

DR. MIASKOWSKI: HERE.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

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MS. BONNEVILLE: ART TORRES.

MR. TORRES: HERE.

MS. BONNEVILLE: CARL WARE.

DR. WARE: HERE.

MS. BONNEVILLE: KAROL WATSON. KEITH YAMAMOTO.

DR. YAMAMOTO: HERE.

MS. BONNEVILLE: THANK YOU.

CHAIRMAN GOLDSTEIN: OKAY. THANK YOU.

WE HAVE A NUMBER OF CONCEPT PLANS AND AMENDMENTS TO CONSIDER TODAY. AND FIRST ONE FOR IS FOR DISC-0. WHO IS DOING THE PRESENTATION PLEASE?

DR. CANET-AVILES: I'LL BE DOING THE PRESENTATION, MR. CHAIRMAN, CHAIR OF THE SUBCOMMITTEE. GREAT. LET ME JUST GO AND SHARE MY SCREEN. CAN YOU SEE MY SLIDES?

MS. BONNEVILLE: YES.

DR. CANET-AVILES: MR. CHAIRMAN, MEMBERS OF THE SCIENCE SUBCOMMITTEE, ON BEHALF OF OUR TEAM AT CIRM, I AM PRESENTING THIS CONCEPT THAT WE WILL BE BRINGING TO THE BOARD FOR APPROVAL THIS MONTH. THIS IS A CONCEPT THAT IS PART OF THE DISCOVERY PILLAR OF PROGRAMS.

SO AS WE ALL KNOW, OUR MISSION IS TO ACCELERATE WORLD-CLASS SCIENCE TO DELIVER

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TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND THE WORLD. AND AS WE KNOW, DESPITE THE PROGRESS IN REGENERATIVE MEDICINE OVER THE PAST DECADE, THERE ARE STILL CRITICAL GAPS IN OUR UNDERSTANDING OF FUNDAMENTAL HUMAN CELL BIOLOGY AND DISEASE THAT AFFECT THE PACE OF SCIENTIFIC DISCOVERY AND PREVENT THE POTENTIAL OF THIS RESEARCH FROM BEING FULLY REALIZED AS THE BASIS FOR NEW THERAPIES AND AS TOOLS FOR BIOMEDICAL INNOVATION.

THE NEW ERA OF TECHNOLOGICAL INNOVATION IN GENE EDITING, SINGLE CELL PROFILING, DATA SCIENCE AND ALSO ENGINEERING IS PRESENTING TO US WITH NEW OPPORTUNITIES FOR ADDRESSING QUESTIONS OF BIOLOGY THAT HAVE REMAINED ELUSIVE. AND ALL THIS WORK HAS LED TO THE NEW STRATEGIC PLAN AND A NEW AND REVISED MISSION STATEMENT REFLECTING THE NEW ERA THAT WE ARE IN.

CIRM SEEKS TO HARNESS ALL OF THESE AND OTHER SYNERGIES THROUGH THIS NEW DISC-0 FOUNDATION AWARDS MECHANISM THAT WOULD BE FOSTERING A ROBUST DISCOVERY ENGINE THAT WILL NOT ONLY OPEN NEW DOORS TO TREATMENTS, BUT ALSO ACCELERATE AND INCREASE THE LIKELIHOOD OF BRINGING REGENERATIVE MEDICINE TREATMENTS TO PATIENTS IN NEED. AND BY

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INCORPORATING PRINCIPLES AND PRACTICES OF DIVERSITY, EQUITY, AND INCLUSION WITHIN THE SCIENCE, THIS PROGRAM ALSO STRIVES TO FOSTER DISCOVERIES THAT WILL EQUITABLY IMPACT PATIENTS IN ALL OUR COMMUNITIES.

CIRM HAS ESTABLISHED, AS WE KNOW, A STRONG TRACK RECORD FOR STRATEGIC INVESTMENTS IN THESE FIVE PILLARS: INFRASTRUCTURE, EDUCATION, DISCOVERY, TRANSLATION, AND CLINICAL. THROUGH CIRM'S NEW STRATEGIC PLAN, WE WILL ENHANCE, ORGANIZE, AND INTERCONNECT CIRM'S PROVEN FUNDING MODEL TO ACHIEVE THE OVERARCHING GOALS. THIS CONCEPT, AS I MENTIONED EARLY ON, IS PRESENTED IN THE CONTEXT OF THE MIDDLE PILLAR WHICH IS THE DISCOVERY PILLAR.

AS A LITTLE BACKGROUND, AND WE HEARD THIS DURING THE WORKSHOP THAT WE HAD A COUPLE OF WEEKS AGO, A WEEK. WE HAVE RECEIVED A LOT OF FEEDBACK FROM AND WE'VE BEEN INFORMED BY MULTIPLE LAYERS OF THE STAKEHOLDERS OVER THE PAST COUPLE OF YEARS. THE DISC-0 CONCEPT HAS ALSO BEEN INFORMED BY THESE STAKEHOLDER DISCUSSIONS AND INPUT THAT STARTED EVEN PRIOR TO PROP 14. AND ONE OF THESE STAKEHOLDER MEETINGS WAS THE BRAINSTORMING NEURODEGENERATION WORKSHOP THAT HAPPENED IN 2019 AND ALSO THE STRATEGIC SCIENTIFIC ADVISORY PANEL IN 2021. AND ACTUALLY WE HAVE ANOTHER VIEW OF WHAT THE OUTCOMES

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OF THESE MEETINGS HAVE BEEN IN ONE OF OUR BLOG POSTS OFFERED BY OUR COLLEAGUE MITRA HOOSHMAND.

THROUGH ALL THESE MEETINGS CIRM RECEIVED BROAD STAKEHOLDER INPUT ABOUT THE NEED FOR CONTINUED RESEARCH OF BASIC MECHANISMS OF STEM CELL BIOLOGY, GENE THERAPY APPROACHES, AND DISEASE BIOLOGY TO IMPROVE THE LIKELIHOOD OF CLINICAL SUCCESS FOR REGENERATIVE MEDICINE. THIS HAS TRANSLATED INTO THE STRATEGIC PLAN THAT I MENTIONED. AND TO REALIZE THE FULL POTENTIAL OF REGENERATIVE MEDICINE FOR SOCIETY, CIRM IS COMMITTED TO PROMOTING SUCCESS OF HIGH RISK, HIGH REWARD PROJECTS.

AND FOR THAT WE ARE PROPOSING THESE NEW OBJECTIVES FOR THE DISCOVERY FOUNDATION AWARDS. AND THE OBJECTIVE OF THE DISCOVERY FOUNDATION AWARDS IS TO SUPPORT RIGOROUS STUDIES ADDRESSING CRITICAL BASIC KNOWLEDGE GAPS IN THE BIOLOGY OF STEM CELLS AND REGENERATIVE MEDICINE APPROACHES AND TO ADVANCE STEM CELL-BASED TOOLS.

PROJECTS FUNDED THROUGH THE FOUNDATION AWARDS SHOULD PROPOSE IMPACTFUL OR INNOVATIVE RESEARCH THAT CULMINATES IN A DISCOVERY OR TECHNOLOGY THAT COULD EITHER ADVANCE OUR UNDERSTANDING OF THE BIOLOGY OR A STEM CELL BIOLOGY THAT'S RELEVANT TO HUMAN BIOLOGY AND DISEASE OR

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ADVANCE THE DEVELOPMENT OR USE OF HUMAN STEM CELLS AS TOOLS FOR BIOMEDICAL INNOVATION OR LEAD TO A GREATER APPLICABILITY OF REGENERATIVE MEDICINE DISCOVERIES TO COMMUNITIES REPRESENTING THE FULLEST SPECTRUM OF DIVERSITY OR ADVANCE THE APPLICATION OF GENETIC RESEARCH THAT IS RELEVANT TO HUMAN BIOLOGY AND DISEASE AS IT PERTAINS TO STEM CELLS AND REGENERATIVE MEDICINE. AND I WILL GO INTO THESE A LITTLE BIT MORE IN THE NEXT SLIDE.

IN TERMS OF ELIGIBILITY FOR THE PROJECT, THESE ELIGIBLE PROJECTS WILL DEFINE AND PROPOSE RESEARCH THAT ADDRESSES A KEY KNOWLEDGE GAP IN OUR UNDERSTANDING OF THE BIOLOGY OR APPLICATION OF STEM CELLS OR PROGENITOR CELLS OR IN THE APPLICATION OF GENETIC RESEARCH AS IT PERTAINS TO STEM CELLS OR REGENERATIVE MEDICINE, AS I WAS SAYING IN THE EARLIER SLIDE.

FOR THE SCOPE OF THIS SOLICITATION, CIRM CONSIDERS GENETIC RESEARCH TO MEAN RESEARCH THAT ALTERS GENOMIC SEQUENCES OF CELLS EITHER BY EDITING, REMOVING, OR ADDING THE DNA SEQUENCES OR INTRODUCES OR DIRECTLY MANIPULATES NUCLEIC ACIDS, SUCH AS MRNA'S OR ASO'S, IN CELLS. WHILE WE DO INCLUDE RESEARCH ON ANIMALS AND ANIMAL CELLS, WE REQUIRE THAT DISCOVERIES MADE IN NON-HUMAN CELLS BE

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VALIDATED WITH A RELEVANT HUMAN CELL EQUIVALENT AS PART OF THE PROPOSED PROJECT.

IN TERMS OF INSTITUTIONAL ELIGIBILITY, ALL CALIFORNIA FOR-PROFIT'S AND NONPROFITS ARE ELIGIBLE TO APPLY. AND PI ELIGIBILITY, THE PRINCIPAL INVESTIGATORS MUST COMMIT AT LEAST 20 PERCENT OF THEIR EFFORT.

NOW, DATA SHARING AND MANAGEMENT PLAN. CONSISTENT WITH THE STRATEGIC PLAN TO LEVERAGE COLLECTIVE KNOWLEDGE TO INSPIRE COLLABORATIVE RESEARCH THAT ADVANCES CALIFORNIAN'S UNMET MEDICAL NEEDS, IF THE BOARD APPROVES THIS CONCEPT, WE ARE PLANNING TO INCORPORATE IN THE APPLICATION SOME GUIDELINES FOR THE DEVELOPMENT AND EXECUTION OF A DATA SHARING AND MANAGEMENT PLAN, EFFECTIVELY CAPTURING SCIENTIFIC KNOWLEDGE AND ENABLING COLLABORATIVE RESEARCH. THESE GUIDELINES AND TEMPLATES ARE THERE TO SUPPORT APPLICANTS IN THE DEVELOPMENT OF SUCCESSFUL RESEARCH PROJECTS AND MAXIMIZE THE COLLABORATIVE POTENTIAL OF CIRM-FUNDED RESEARCH. WE ARE ALSO GOING TO PROVIDE GUIDELINES FOR ALLOCATION OF FUNDS FOR PERSONNEL AND/OR ACTIVITIES RELATED TO MANAGING AND SHARING THE DATA.

AND FOR THE DATA SHARING AND MANAGEMENT PLAN, APPLICANTS WILL ALSO BE REQUIRED TO ADHERE TO

THE FAIR DATA SHARING PRINCIPLES OF ACCESSIBLE, INTEROPERABLE, AND REUSABLE DATA FOR SCIENTIFIC RESEARCH AND THERAPEUTIC DEVELOPMENT.

IN TERMS OF BUDGET, CIRM WILL FUND DIRECT PROJECTS COSTS OF UP TO $1 MILLION PER AWARD FOR UP TO THREE YEARS DURATION. AND OF NOTE, WE HAVE CHOSEN TO PROVIDE THE DIRECT COSTS IN THIS SLIDE, BUT FOR INDIRECT COSTS, WHICH IS HIGHLIGHTED IN THE BOTTOM THERE, THE COSTS PER PROJECT, AS YOU KNOW, PER INSTITUTION OF INDIRECTS, BUT THE AVERAGE IS ABOUT $1.5 MILLION FOR THREE YEARS PER INSTITUTION. TAKING THIS INTO ACCOUNT, AND IF THE OPPORTUNITY IS AVAILABLE ONE TO TWO TIMES A YEAR, WE ESTIMATE, BASED ON THE PREVIOUS BASIC DISC PILLAR PROGRAMS AND OTHER INFORMATION THAT WE BENCHMARKED TO THAT, WE ESTIMATE AN APPROXIMATE OF 12 TO 14 AWARDS WITH AN ESTIMATE OF $20 MILLION PER ROUND.

AND THIS WILL BE THE REQUEST THAT WE WILL BRING TO THE BOARD ON THE 24TH OF MARCH, AND WE WILL BE REQUESTING THIS CONCEPT FOR APPROVAL. AND THEN WITH THIS, I'M DONE WITH MY PRESENTATION AND I WILL WELCOME ANY QUESTIONS THAT YOU MIGHT HAVE.

CHAIRMAN GOLDSTEIN: THANK YOU. EXCELLENT PRESENTATION. LET'S HAVE QUESTION OR DISCUSSION FROM THE MEMBERS OF THE SUBCOMMITTEE PLEASE.

DR. WARE: I'D LIKE TO RAISE A QUESTION ABOUT THE PERCENT EFFORT. ARE WE GOING TO BE EXCLUDING MID-CAREER AND SENIOR INVESTIGATORS BASED ON A 20-PERCENT COMMITMENT TIME?

DR. CANET-AVILES: IF THEY CANNOT COMMIT THAT PERCENTAGE OF TIME, THEN WE COULD BE EXCLUDING THEM, YES. IT'S NOT ABOUT WHICH STAGE IN THEIR CAREER THEY ARE. IT IS ABOUT WHETHER THEY CAN COMMIT THE APPROPRIATE TIME THAT WE ARE REQUIRING FOR THIS TYPE OF PROJECTS.

DR. WARE: I'M JUST SAYING THAT IN TERMS OF MANY OF OUR MOST SUCCESSFUL MID-TERM AND SENIOR INVESTIGATORS, A TYPICAL NIH GRANT HAS THEM AT 5 PERCENT BECAUSE THEY'RE COMMITTED TO THREE OR FOUR OTHER GRANTS AND MAYBE OTHER OPPORTUNITIES.

DR. CANET-AVILES: THEY COULD COME IN AS ANOTHER MEMBER OF THE TEAM INSTEAD OF BEING THE PI AND LEAVE THE PI TO SOMEONE MORE JUNIOR THAT MIGHT HAVE A CHANCE TO COME IN AND LEAD THE PROJECT. WE ALSO WANT TO INCENTIVIZE YOUNGER INVESTIGATORS TO GET INTO LEADING POSITIONS.

DR. WARE: CERTAINLY.

DR. LEVITT: IS IT ACCEPTABLE TO HAVE MULTIPLE PI'S? AND, THEREFORE, IF YOU HAVE, LET'S SAY, TWO MPI'S, EACH ONE AT 10 PERCENT, THAT'S A

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TOTAL OF 20 PERCENT.

CHAIRMAN GOLDSTEIN: INTERESTING QUESTION.

DR. CANET-AVILES: I CAN'T RECALL -- GO AHEAD.

DR. LEVITT: I WAS GOING TO SAY IF THEY'RE MPI'S, WHICH REQUIRE, CERTAINLY AT NIH WHAT'S REQUIRED IS AN MPI PLAN: WHO'S RESPONSIBLE FOR WHAT? HOW ANY DISAGREEMENTS WOULD BE MANAGED, ET CETERA. SO THERE'S A SEPARATE PLAN FOR MPI'S, BUT THEY'RE BECOMING MORE AND MORE POPULAR. JUST LIKE SHARING A FIRST AUTHORSHIP, RIGHT, THAT PRECEDED THIS, BUT IT'S BECOMING MORE POPULAR. AND ALSO HAS A POSSIBILITY OF PROVIDING A TETHERED WAY OF PROFESSIONAL DEVELOPMENT FOR JUNIOR AND SENIOR INVESTIGATORS.

DR. CANET-AVILES: I THINK ONE OF THE THINGS THAT I'VE SEEN IN SOME OTHER AGENCIES, THEY ARE INCENTIVIZING THESE MULTIDISCIPLINARY TYPE OF TEAMS WITH SOMEBODY THAT MIGHT BE A MORE JUNIOR PI, BUT THEN NEEDS TO HAVE ANOTHER CO-PI THAT IS MORE SENIOR THAT COULD BE AT A LESSER PERCENTAGE. THOSE ARE SOME THINGS, AND I THINK OUR PRESIDENT MARIA MILLAN HAS HER HAND RAISED. MARIA.

DR. MILLAN: I ACTUALLY WAS GOING TO ASK IF GIL SAMBRANO AND JENN LEWIS CAN SPEAK TO WHAT OUR

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CURRENT POLICY IS BECAUSE IF IT IS SOMETHING TO CONSIDER, IT WOULD REQUIRE A CHANGE OR AN AMENDMENT TO OUR POLICY. GIL.

DR. SAMBRANO: I'M HAPPY TO SPEAK TO THAT. SO CURRENTLY THE WAY OUR GRANTS ADMINISTRATION POLICY IS STRUCTURED, WE ONLY ALLOW FOR ONE PI. AND PART OF IT IS BECAUSE WE DEFINE THE PI TO BE THE SINGLE PERSON WHO IS RESPONSIBLE FOR THE CONDUCT OF THE AWARD UNDER THOSE POLICIES.

AND SO THE PERCENT EFFORT IS TIED TO THAT ONE INDIVIDUAL. THAT DOESN'T, OF COURSE, PREVENT OTHERS FROM PARTICIPATING AND SHARING IN AND CONTRIBUTING TO THE PROJECT IN SOME WAY. BUT THE 20 PERCENT IS PRETTY MUCH FIXED ON A SINGLE PERSON AT THIS TIME.

DR. CANET-AVILES: THANK YOU, GIL.

DR. LEVITT: SO I UNDERSTAND THAT IT WOULD HAVE TO BE CONSIDERED AS A CHANGE OF POLICY. BUT GIVEN THE MULTIDISCIPLINARY NATURE OF ALL THESE PROJECTS, THEY'RE ALL MULTIDISCIPLINARY NOW, AND THE OPPORTUNITY TO BE MORE INCLUSIVE, IT'S SOMETHING TO CONSIDER. AND THEN THERE COULD STILL BE A RULE ABOUT, IF IT'S A SINGLE PI, IT'S 20-PERCENT EFFORT. IF IT'S MPI, IT'S EQUIVALENT -- THAT'S WHAT NIH EXPECTS -- EQUIVALENT EFFORTS BETWEEN THE MPI'S

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BECAUSE YOU CAN'T HAVE ONE AT 15 AND ONE AT 5. THAT'S NOT AN MPI SITUATION. THAT'S A SINGLE PI AND A COINVESTIGATOR. BUT YOU COULD REQUIRE IT TO BE ADDING TO 20 PERCENT.

THE OTHER THING THAT IS DONE IS THERE'S ONE OF THE TWO IS DESIGNATED AS THE CORRESPONDING PI. AND THAT'S WHO THE BUSINESS IS DONE WITH AND IS ULTIMATELY RESPONSIBLE FOR THE FINANCIAL AND ADMINISTRATIVE OVERSIGHT.

DR. CANET-AVILES: QUESTION. THANK YOU, PAT. MY QUESTION TO GIL AND JENN COULD BE DO WE NEED TO AMEND THE GAP TO ALLOW FOR THIS?

DR. SAMBRANO: NO. IN ORDER TO ALLOW MULTIPLE PI'S, YOU WOULD. I THINK THE QUESTION IS DO YOU WANT TO ADJUST THE PERCENT EFFORT FOR THE WAY WE CURRENTLY DEFINE THE PI, WHICH IS PERHAPS AKIN TO WHAT IS THE ADMINISTRATIVE PI THAT WE HOLD ACCOUNTABLE FOR ALL THE AWARD AND COMMUNICATION.

SO AS MENTIONED, IF YOU LOWER, FOR EXAMPLE, THE PERCENT EFFORT TO 10 PERCENT, YOU CAN BRING OTHERS ON BOARD TO BE SORT OF A PSEUDO CO-PI. WE DON'T NECESSARILY HAVE A SPECIFIC DEFINITION FOR ONE, BUT OTHERS THAT CAN CONTRIBUTE AND COLLABORATE IN A SIMILAR WAY.

DR. CANET-AVILES: COULDN'T WE GET INTO

THE POTENTIAL ISSUE OF HAVING THEN PEOPLE THAT ARE NOT COMMITTED, THEY ARE SENIOR AND NOT COMMITTED TO THE EXTENT THAT WE NEED FOR THE PROJECT, AND THEN WE KIND OF DILUTE THE EFFORT IN TERMS OF LEADERSHIP ON A PROJECT OF IMPORTANCE. COULD THIS BE A PROBLEM?

DR. LEVITT: I DON'T THINK IT'S A PROBLEM BECAUSE IN THE BUDGET JUSTIFICATION I ASSUME THAT IN PERSONNEL THAT YOU HAVE TO DESCRIBE WHAT EACH KEY PERSONNEL IS RESPONSIBLE FOR ON THE PROJECT. AND IF IT'S UNSATISFACTORY -- IF IT SEEMS LIKE, OH, ONE WILL BE THE OVERSIGHT PERSON AND THE OTHER WOULD DO ALL OF THE RESEARCH. I MEAN I HAVEN'T SEEN THAT AS A PROBLEM.

I DON'T WANT TO LENGTHEN THIS OR CONFUSE IT. MAYBE WE CAN TAKE IT UP SOME OTHER TIME, BUT I DO THINK THAT IT'S BECOMING MORE AND MORE COMMON BECAUSE A SINGLE PI DOES NOT HAVE THE ABILITY TO REALLY OVERSEE AN ENTIRETY OF THESE MULTIDISCIPLINARY PROJECTS. SO YOU CAN CALL THEM A CO-PI, I SUPPOSE, BUT THAT DIMINISHES THEIR TITLE OR ROLE THAT THEY'RE REALLY PLAYING IN THE PROJECT. AND, AGAIN, I THINK IT CREATES AN OPPORTUNITY TO BE MORE INCLUSIVE OF DEVELOPING INTERDISCIPLINARY PARTNERSHIPS.

DR. CANET-AVILES: OKAY. MARIA.

CHAIRMAN GOLDSTEIN: WE HAVE TWO HANDS. MARIA, GO.

DR. MILLAN: IF THE BOARD, AFTER THIS DISCUSSION, WISHES US TO GO BACK AND PROPOSE HOW THIS COULD BE DONE, THAT'S CERTAINLY SOMETHING WE CAN DO. AND I THINK GIL GAVE A GOOD EXAMPLE OF HOW THAT CAN BE DONE IN OUR CURRENT POLICY.

DR. MIASKOWSKI, I DIDN'T MEAN TO RAISE MY HAND AT THE SAME TIME.

DR. MIASKOWSKI: THAT'S OKAY. I'D LIKE TO SPEAK IN SUPPORT OF PAT'S IDEA. I'VE BEEN INVOLVED NOT IN OBVIOUSLY STEM CELL BIOLOGY PROJECTS, BUT IN OTHER PROJECTS WITH MULTIPLE PI'S. AND AS PAT SAID, THERE IS A CONTACT PI THAT IS THE ADMINISTRATIVE PERSON. BUT THE MULTIPLE PI PLAN, I THINK, MIGHT ADDRESS ROSA'S CONCERN WHERE PEOPLE ACTUALLY SPELL OUT THE RATIONALE FOR NEEDING TWO PI'S, AND THEN THE RESPONSIBILITIES OF EACH OF THOSE PI'S IS LISTED. AND THEN THE REVIEW COMMITTEE CAN ADJUDICATE WHETHER OR NOT THE PERCENT EFFORT THAT'S APPLIED TO THOSE MULTIPLE PI'S IS ACCURATE TO GET THE WORK DONE.

IN MY EXPERIENCE DOING THIS, IT REALLY, REALLY ENRICHES THE PROJECT BECAUSE YOU BRING PEOPLE WITH TWO OFTEN DIVERSE BACKGROUNDS THAT ARE GOING TO BE GREATER SYNERGISTICALLY IN TERMS OF THE SUM OF

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THE PARTS. IT REALLY, REALLY WORKS QUITE WELL. SO I WOULD SUPPORT WHAT PAT SAID.

MS. BONNEVILLE: LARRY, YOU'RE ON MUTE.

CHAIRMAN GOLDSTEIN: I WAS JUST GOING TO MENTION THAT CIRM HAS HAD THESE LARGE PERCENT EFFORT REQUIREMENTS FOR A NUMBER OF YEARS. SO I THINK IN ONE SENSE IT'S BEEN WORKING OKAY. THERE HASN'T BEEN A PUSH TO MAKE A BIG CHANGE UNTIL THIS CONVERSATION, I THINK.

ON THE OTHER HAND, I CAN SEE THE VALUE OF DOING SOME SORT OF EXPERIMENT ON OUR USER COMMUNITY TO SAY TRY IT OUT ON A PILOT BASIS, SEE WHETHER WE GET THINGS THAT ARE SENSIBLE AND THAT REVIEWERS AGREE ARE SENSIBLE PLANS THAT PROVIDE MAXIMAL ENGAGEMENT AND COMMITMENT FROM THE PI'S OF THE PROJECT. THAT'S WHAT I THINK WE ARE WORRIED ABOUT. WE DON'T WANT A SITUATION WHERE SOMEBODY SLIDES IN UNDER THE LIMIT AND ISN'T REALLY VERY COMMITTED.

I GUESS THE QUESTION I WOULD HAVE FOR ROSA AND THE TEAM IS HOW HARD WOULD IT BE TO ENGINEER A SINGLE CYCLE OF APPLICATIONS WHERE WE TRIED THIS OUT AND GOT A SENSE OF IS THERE A LOT OF DEMAND FOR IT, AND THEN WHAT SORTS OF ARRANGEMENTS DO WE SEE?

DR. CANET-AVILES: I DON'T THINK IT WOULD BE VERY HARD. I DON'T THINK IT COULD BE VERY HARD.

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I THINK WE HAVE HEARD ENOUGH EXAMPLES AND FEEDBACK DURING THIS CALL THAT WE CAN ACTUALLY DEVELOP THE APPLICATION AND THE REVIEW CRITERIA WITH OUR REVIEW OFFICE THAT WILL ENABLE TO ENGINEER A PILOT CYCLE THAT COULD IMPLEMENT THESE CHANGES. I THINK IT COULD BE INTERESTING TO SEE THE OUTCOMES, AND IT COULD BE VERY INFORMATIVE FOR US. MARIA, YOU AGREE AS WELL?

DR. MILLAN: I THINK THAT, JUST BY NATURE IF WE GAVE A CHOICE, IT WOULD JUST GET PILOTED BECAUSE YOU'D SEE WHAT THE UTILIZATION IS AND YOU'D SEE HOW THEY FAIR IN THE REVIEW PROCESS. AND THEN IF DOESN'T WORK OUT WELL, IT JUST DEFAULTS TO OUR ORIGINAL. SO I AGREE AND I WOULD DEFER TO GIL, WHO'S GOING TO BE IN CHARGE OF MAKING THIS HAPPEN. AND THEN, OF COURSE, IT SOUNDS LIKE IT'S COMPATIBLE WITH THE GAP, BUT I'D LOVE TO HEAR IF JENN LEWIS HAS ANY OTHER THOUGHTS ON THAT. GIL.

DR. SAMBRANO: DID YOU WANT TO GO TO JENN OR TO ME?

DR. MILLAN: JUST WANTED TO GET YOUR TAKE AS TO WHETHER IN SOME FORMAT THIS COULD BE SOMETHING MADE AVAILABLE IN ONE WAY OR THE OTHER, EITHER A PILOT OR GIVING THE APPLICANTS A CHOICE TO CHOOSE -- TO TAKE THE 20 PERCENT ON THEIR OWN OR TAKE ANOTHER

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FORMAT, TBD WHAT THAT LOOKS LIKE, WE'LL BRING IT BACK TO THE BOARD ONCE THAT'S SETTLED. BUT DOES THAT SEEM LIKE A FEASIBLE APPROACH?

DR. SAMBRANO: I THINK WE NEED TO LOOK AT IT CAREFULLY BECAUSE I'M ACTUALLY NOT SURE WHAT IMPLICATIONS WE HAVE IN THE GRANTS ADMINISTRATION POLICY FOR A PI. BECAUSE WE DEFINE PRETTY SPECIFICALLY WHAT A PI IS. AND IT CURRENTLY DOES NOT ALLOW SPLITTING THAT RESPONSIBILITY OUT. SO IT MAY REQUIRE POTENTIALLY A MODIFICATION OF THE GAP. SO WE NEED TO LOOK INTO IT TO SEE HOW DOABLE IT IS.

CHAIRMAN GOLDSTEIN: JENNIFER LEWIS, MARIA THOUGHT YOU MIGHT KNOW OFF THE TOP OF YOUR HEAD THE ANSWER TO GIL'S QUESTION.

MS. LEWIS: I NEED TO LOOK A LITTLE CLOSER. FROM JUST REVIEWING THE GAP, I THINK WE HAVE FLEXIBILITY IN PROVIDING THE OPTION IN THE PROGRAM ANNOUNCEMENT TO TEST IT OUT. I DO THINK WE MIGHT WANT TO JUST AFTER THIS MEETING GO BACK AND LOOK AT THE LANGUAGE AND HAVE THE LEGAL TEAM WEIGH IN ON WHETHER THAT'S -- BUT FROM MY VIEW RIGHT NOW, I THINK THERE'S FLEXIBILITY TO PUT IT IN THE PROGRAM ANNOUNCEMENT.

DR. CANET-AVILES: OKAY. SO I THINK WHAT WE WILL DO IS WE WILL COME BACK TO THE BOARD WITH

LIKE WHETHER THERE IS FLEXIBILITY PROVIDING THIS IN THE PROGRAM ANNOUNCEMENT CORROBORATED FROM THE LEGAL PERSPECTIVE, OR LOOK AT THIS AND GET BACK BY E-MAIL, NO? OKAY.

CHAIRMAN GOLDSTEIN: THAT SOUNDS REASONABLE TO ME. SO THE PROPOSAL WOULD BE THE GRANTS ADMINISTRATION TEAM WILL HAVE A LOOK AT THE LANGUAGE, SEE WHETHER THIS IS SOMETHING WE COULD OFFER AS AN EXPERIMENT IN THIS ROUND. AND WHEN WE VOTE ON THIS PARTICULAR CONCEPT PLAN, WHICH I THINK WE NEED TO DO, WE WILL DO IT WITH THE PROVISO THAT THERE IS THIS POSSIBILITY THAT THERE WOULD BE SORT OF A LAST-MINUTE ALTERATION TO OFFER A PILOT PROGRAM FOR MULTIPLE PI'S ADDING UP TO 20 PERCENT. HAVE I CAPTURED THE ESSENCE OF THIS?

DR. CANET-AVILES: EXCELLENT. YES, YOU DID.

MS. BONNEVILLE: LARRY, I'LL JUST NOTE THAT IF WE CAN GET THAT SETTLED BEFORE WE GO TO THE BOARD IN MARCH BECAUSE, REMEMBER, THIS WILL MOVE TO THE BOARD IN MARCH, AND THEN WE CAN MAKE THE FINAL VOTE FOR IT AT THE MARCH BOARD MEETING.

CHAIRMAN GOLDSTEIN: SOUND FEASIBLE, GUYS? GIL? JENNIFER? MARIA?

DR. SAMBRANO: YES FROM MY END.

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CHAIRMAN GOLDSTEIN: THAT'S INTERESTING. ANY OTHER DISCUSSION ITEMS?

I WANT TO JUST ADD SOMETHING IN A REQUEST. I LOOKED AT THE DATA SHARING PLAN. AS YOU KNOW, I'VE HAD AN INTEREST IN THIS PARTICULAR ISSUE. I WONDER IF IT'S POSSIBLE TO OFFER LINKS TO OUR GRANTEE COMMUNITY TO THE NIH POLICIES THAT GIVE SOME EXAMPLES OF HOW THEY MIGHT CARRY THESE THINGS OUT. I DO WORRY THAT IT'S A LITTLE TOO OPEN-ENDED, AND IT WOULD BE HELPFUL IF WE GAVE SOME ADDITIONAL ADVICE TO OUR APPLICANTS.

DR. CANET-AVILES: YES. I HAD A COUPLE OF SLIDES TO PROVIDE A LITTLE BIT MORE DETAIL ABOUT THE TYPE OF DATA ELEMENTS THAT WE ARE CONSIDERING, AND IT'S ALL BEEN ALIGNED WITH THE NIH NEW POLICIES THAT ARE COMING INTO EFFECT IN 2023 AND HAVE BEEN UNDER DISCUSSION AND REVIEW FOR THE LAST THREE YEARS, I THINK. SO WE'VE BEEN CAREFULLY LOOKING AT THIS AND EVALUATING THEM, AND THAT'S WHAT WE WOULD BE BASING.

SO WE COULD -- ONE CONUNDRUM HERE WOULD BE IF WE LINK TO THEIR WHOLE POLICIES, IT COULD BE CONFUSING BECAUSE NIH IS VERY DIFFERENT INSTITUTES. WE HAVE A BIT MORE HOMOGENEITY IN WHAT WE CAN OFFER TO OUR GRANTEES. SO WOULD WE WANT TO PERHAPS PROVIDE A PDF THAT OUTLINES WHAT WE ARE THINKING

ABOUT INSTEAD OF LINKING TO THEM? I'M JUST SUGGESTING.

AND WE HAVE -- MARIA AND DAVID HIGGINS, I DON'T KNOW WHO WAS FIRST. MARIA, I THINK, WAS FIRST.

DR. MILLAN: I'LL LET THE CHAIR CHOOSE. I WANTED TO SUGGEST SOMETHING AFTER DR. HIGGINS SPEAKS IN TERMS OF THAT SPECIFIC QUESTION. AND WE CAN EITHER DO IT NOW OR AT THE TIME THAT GIL PRESENTS THE CONCEPT CHANGES BECAUSE THE DATA SHARING IS SOMETHING THAT'S BEING PROPOSED ACROSS THE PROGRAM. BUT I'LL JUST YIELD TO DR. HIGGINS FIRST. AND THEN THAT'S ON THE TABLE. WE DO HAVE SOME SUPPLEMENTAL INFORMATION TO JUST ILLUSTRATE HOW THIS COULD BE IMPLEMENTED.

CHAIRMAN GOLDSTEIN: GOOD. DAVID, GO AHEAD.

DR. HIGGINS: THANK YOU, PRESIDENT MILLAN. I JUST WANTED TO CHIME IN AND SECOND OR BACK UP WHAT LARRY WAS JUST SAYING ABOUT US SORT OF GIVING PEOPLE SOME INFORMATION ABOUT HOW THEY COULD MOST SUCCESSFULLY CRAFT THEIR APPLICATION. I JUST WANTED TO SHARE WITH YOU THAT MY KNOWLEDGE, GENERAL KNOWLEDGE, OF CIRM PRIOR TO EVEN BEING INVOLVED WITH CIRM WAS THAT THEY WERE AN INSTITUTION THAT THEIR

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GOAL WASN'T TO TRICK YOU AND MAKE YOU FAIL. THE GOAL WAS TO FIND OUT WHAT CIRM COULD DO TO MAKE YOU SUCCEED TO GET MONEY.

AND I THINK THAT THAT PHILOSOPHY IS VERY UNUSUAL, VERY UNIQUE, AND EXTREMELY POWERFUL. AND I THINK THAT'S WHAT LARRY BASICALLY IS SAYING, NOT TO PUT WORDS IN YOUR MOUTH, AND I JUST WANTED TO SECOND THAT. I THINK THAT'S REALLY, REALLY WHAT SETS US APART FROM OTHER PLACES THAT GRANT MONEY.

CHAIRMAN GOLDSTEIN: THANK YOU, DAVID.

DR. MILLAN: WE HAVE A GENERAL SCHEME THAT WE CAN SHARE AT ANY TIME, DR. GOLDSTEIN, WHENEVER YOU THINK IT'S APPROPRIATE.

CHAIRMAN GOLDSTEIN: SO IT WOULD APPLY TO DISC-0 AS WELL AS 2 AND THE TRAN, AND CLIN-1 AND 2 BECAUSE THEY'RE LISTED SEPARATELY IN THE AGENDA?

DR. MILLAN: IF IT'S RELEVANT TO THE SUBCOMMITTEE DELIBERATIONS ON THE DISC-0, THIS COULD BE A FINE TIME FOR OUR TEAM TO PRESENT THIS SO THAT YOU CAN KIND OF LOOK AT DISC-0 HOLISTICALLY. IT'S ALSO RELEVANT TO THE CONCEPT CHANGES. SO ROSA HAS SOME SUPPLEMENTARY DISCUSSION SLIDES THAT COULD HELP IN THAT IF THAT'S WHAT YOU WOULD LIKE TO DO.

CHAIRMAN GOLDSTEIN: THANK YOU. THAT SOUNDS LIKE A GREAT IDEA TO ME, MARIA. THANK YOU

VERY MUCH. ROSA.

DR. CANET-AVILES: I'M TRYING TO LEARN FROM LARRY. OKAY.

SO THIS IS THE PHASED APPROACH IMPLEMENTATION OF DATA SHARING. AND AS YOU KNOW, CIRM HAS IN RECENT YEARS ALREADY INCORPORATED DATA MANAGEMENT PLANS IN OUR GRANT PROPOSALS. THIS WAS FIRST IMPLEMENTED DURING THE EMERGENCY COVID PROGRAM AND HAS HIGHLIGHTED THE IMPORTANCE OF COLLABORATIVE RESEARCH AND DATA SHARING, WHICH WAS VERY CRITICAL AT THE TIME.

SINCE THEN, ONE OF THE THINGS THAT WE HAVE REALIZED IS THAT THERE IS VARIABILITY IN THE PLANS THAT ARE COMING IN. AND THAT MADE US REALIZE THAT WE NEED TO BE MORE CLEAR IN THE GUIDELINES AND TEMPLATES THAT WE PROVIDE TO BE ABLE TO HELP OUR GRANTEES BETTER TO BE SUCCESSFUL.

SO WITH THE BOARD'S APPROVAL, IF WE APPROVE THIS, WE COULD BE IMPLEMENTING A SECOND PHASE DESIGN TO SOLVE THESE ISSUES BY PROVIDING GUIDELINES FOR DATA MANAGEMENT AND SHARING. SO CIRM COULD BE PROVIDING IN THE RFA CLEAR INSTRUCTIONS TO RESEARCHERS TO PROVIDE A PLAN FOR HOW SCIENTIFIC DATA WILL BE PRESERVED AND SHARED IN ACCORD WITH THE FAIR PRINCIPLES. AND WE WOULD ALSO, AS I MENTIONED

EARLIER ON, ALLOCATE FUNDS IN THE PROPOSED BUDGET FOR PERSONNEL AND/OR ACTIVITIES RELATED TO MANAGING AND SHARING OF THIS DATA. AND I WILL PROVIDE A LITTLE BIT MORE DETAIL IN THE NEXT SLIDE.

APPLICATIONS, THROUGH THE PROGRAM ANNOUNCEMENT, WE WOULD HAVE AN APPLICATION THAT COULD PROVIDE TWO TYPES OF TEMPLATES. ONE WOULD BE FOR THE DATA MANAGEMENT AND SHARING PLAN AND THEN ONE FOR ALLOWABLE COSTS. WE WOULD ALSO BE PROVIDING A LIST OF ESTABLISHED AND AVAILABLE DATA REPOSITORIES FOCUSING ON RESEARCHOMICS DATASETS, AND ALSO A LIST OF TRAINING AND TUTORIALS, LINKS TO DIFFERENT TUTORIALS AND TRAINING THAT HAVE BEEN MADE AVAILABLE THROUGH THE FEDERAL GOVERNMENT AND OTHER INSTITUTIONS THAT COULD BE HELPFUL WHEN USING THESE REPOSITORIES OF DATA.

THROUGH THE GRANTS WORKING GROUP REVIEW, THERE COULD BE CLEARLY DEFINED CRITERIA FOR REVIEW OF THE STRENGTH OF THE DATA MANAGEMENT PLAN. AND IN TERMS OF ACCOUNTABILITY, INCORPORATION OF THE DATA SHARING AND MANAGEMENT PLAN INTO THE AWARD MILESTONES.

PHASE II COULD THEN START. THE TIMING FOR THIS IS TBD. OF COURSE, VERY DEPENDENT ON THE DISCUSSIONS AND FEEDBACK FROM THE BOARD, BUT

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COORDINATE AND SHAPE BY CIRM FUTURE DATA AND CONSORTIA PROGRAMS.

IN THIS NEXT SLIDE WE HAVE BASICALLY SOME EXAMPLES OF WHAT WE COULD BE ASKING IN A PLAN. MANY THESE THINGS WILL BE INCORPORATED AND HAVE FUNDING THROUGH THE AWARD BASICALLY TO ENABLE THESE AND THE PERSONNEL THAT WOULD BE RESPONSIBLE TO ENSURE ADHERENCE TO THESE REQUIREMENTS. SOME COULD BE PROVIDING FUNDS THROUGH THE AWARD.

SO THE PROPOSED ELEMENTS OF THE DATA TYPE AND STANDARDS, THAT WOULD BE DESCRIBING THE SCIENTIFIC DATA TO BE MANAGED AND PRESERVED AND SHARED TOGETHER WITH AN INDICATION OF WHAT TYPE OF STANDARDS WILL BE APPLIED TO THE SCIENTIFIC DATA AND ASSOCIATED METADATA, FOR EXAMPLE.

IN TERMS OF RELATED TOOLS, WE WOULD BE ASKING TO PROVIDE AN INDICATION OF WHETHER SPECIALIZED TOOLS WOULD BE NEEDED TO ACCESS OR MANIPULATE SHARED SCIENTIFIC DATA TO SUPPORT THE REPLICATION OR THE REUSE. AND WE WOULD BE ASKING, IF APPROPRIATE, IF THEY NEEDED TOOLS AND SOFTWARE, WHICH ONES WOULD BE.

IN TERMS OF DATA PRESERVATION, ACCESS AND TIME LIMES FOR ACCESS, THIS COULD INCLUDE PLANS AND TIMELINES FOR DATA PRESERVATION AND ACCESS,

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INCLUDING THE NAME OF THE REPOSITORY. THE REPOSITORIES FOR SCIENTIFIC DATA AND METADATA ARISING FROM THE PROJECT WOULD BE ARCHIVED. IN THIS CONTEXT, CIRM COULD BE PROVIDING SUPPLEMENTARY INFORMATION TO ASSIST IN SELECTING SUITABLE REPOSITORIES FOR SCIENTIFIC DATA RESULTING FROM THE FUNDED RESEARCH.

AND MY COLLEAGUE, SHYAM PATEL, HAS SOME SUPPLEMENTARY INFORMATION WITH REGARDS TO THIS IN TERMS OF WHAT WE COULD BE PROVIDING AND WHAT HAS ALREADY BEEN DONE THROUGH CIRM. WHAT SOME OF OUR GRANTEES HAVE BEEN USING IN TERMS OF REPOSITORIES.

IN TERMS OF REUSE, DISTRIBUTION OF DATA CONSIDERATIONS, A DESCRIPTION, WE WOULD BE ASKING FOR A DESCRIPTION OF ANY APPLICABLE PRACTICE THAT COULD AFFECT SUBSEQUENT ACCESS, DISTRIBUTION, OR REUSE OF SCIENTIFIC DATA RELATED, FOR EXAMPLE, TO INFORMED CONSENT, PRIVACY, PROTECTIONS, OR WHETHER ACCESS TO THE SCIENTIFIC DATA DERIVED FROM HUMANS COULD BE CONTROLLED, AND ANY OTHER RESTRICTIONS OR CONSIDERATIONS.

AND THEN TERMS OF GOVERNANCE AND DATA SHARING MANAGEMENT, WE WOULD BE ASKING TO INDICATE HOW COMPLIANCE WITH THE PLAN WOULD BE MONITORED AND MANAGED AND THE OVERSIGHT, ET CETERA. AS I

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MENTIONED, AGAIN, TO ENABLE THESE AND THE PERSONNEL THAT WOULD BE RESPONSIBLE FOR ENSURING THE ADHERENCE TO ALL THESE REQUIREMENTS, CIRM COULD BE PROVIDING FUNDS THROUGH THE AWARD. I HOPE THIS IS HELPFUL.

CHAIRMAN GOLDSTEIN: I THINK THAT'S GREAT, ROSA. I THINK IT'S A TERRIFIC STEP AHEAD. I THINK IT WILL HELP OUR GRANTEES AND APPLICANTS WITH MANAGING THIS. OBVIOUSLY SOME TYPES OF DATA ARE GOING TO BE MUCH MORE STRAIGHTFORWARD THAN OTHERS. OMICS DATA WE ALREADY KNOW HOW TO DO. IMMUNOFLUORESCENCE MICROSCOPY EXPERIMENTS, IT'S NOT COMPLETELY OBVIOUS THAT WE YET KNOW EXACTLY HOW TO HANDLE THOSE DATA, BUT MAYBE WE WILL LEARN.

I WONDER IF I COULD ASK KEITH, IF HE'S STILL ON THE LINE, WHO'S VERY IN TOUCH WITH THE NIH, IS THERE ANYTHING THAT'S BEEN LEARNED AT THE NIH ABOUT HOW TO MANAGE THESE SORTS OF PLANS, OR HAVE THERE BEEN ANY TERRIBLE MISTAKES WE SHOULD KNOW ABOUT?

DR. YAMAMOTO: I DON'T THINK SO. THE PLAN IS REALLY SET TO BE IMPLEMENTED IN 2023. AND SO WE HAVE ALL SEEN THE PROPOSALS THAT HAVE BEEN PUT FORWARD. DATA SHARING ON THE CLINICAL TRIAL SIDE REMAINS, IN MY VIEW, HOPELESSLY WEAK. ACTUALLY LAUNCHED IN THE FIRST DATA SHARING PLAN PUT FORWARD

IN 2003 FOR NIH. THERE'S AN ADVISORY THAT CLINICAL TRIAL DATA -- PROPOSALS FOR CLINICAL TRIALS FROM NIH MUST BE ACCOMPANIED BY A DATA SHARING PLAN, BUT NOT ANY REQUIREMENT FOR DATA SHARING. AND THAT LANGUAGE HAS PERSISTED SINCE 2003 THROUGH EIGHT VERSIONS UNTIL WHAT IS NOW CALLED THE FINAL PLAN.

AND SO I DON'T KNOW HOW MUCH WE'RE GOING TO LEARN FROM THERE. BUT THE OVERALL DATA SHARING SCHEME WILL BE IMPLEMENTED IN '23, AND WE'LL BEGIN TO SEE -- AND ONLY THEN WILL WE REALLY BE ABLE TO SEE WHETHER THERE ARE SOME FLAWS THAT NEED TO BE ADDRESSED.

CHAIRMAN GOLDSTEIN: GREAT. THANK YOU, KEITH.

ROSA AND TEAM, WE DO INTEND OUR PLAN TO APPLY TO CLINICAL TRIAL DATA ULTIMATELY, DON'T WE?

DR. CANET-AVILES: YES. THIS DATA SHARING PLAN -- AND I SHOULD ACTUALLY DEFER TO MY COLLEAGUE DR. CREASEY PERHAPS. ABLA.

DR. CREASEY: THANK YOU, ROSA. THANK YOU, LARRY.

SO WE ARE STILL THINKING ABOUT THE DATA SHARING PLAN FOR CLINICAL, BUT WE KNOW, FOR EXAMPLE, OUR SICKLE CELL GRANTS THAT ARE WORKING CLOSELY WITH NIH AND NHLBI, THEIR DATA ARE LIKELY TO GO INTO

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BIODATA CATALYST, WHICH IS, AGAIN, AN IMPORTANT DATABASE FOR NIH. BUT WE ARE STILL, AGAIN, THINKING ABOUT IS THIS LIKE WITH ONE REPOSITORY AND MAYBE THERE ARE OTHERS. SO IT'S STILL BEING STUDIED.

CHAIRMAN THOMAS: THANK YOU. PAT LEVITT, YOU HAVE A QUESTION OR COMMENT.

DR. LEVITT: YEAH. I WAS JUST GOING TO COMMENT. SO THE OMICS DATA ARE OBVIOUSLY THE MOST RELEVANT AND EASIEST TO COMPLY WITH, AND IT'S BEEN RELATIVELY SUCCESSFUL. I CAN TELL YOU JUST FROM MY OWN EXPERIENCE, OTHER KINDS OF DATA, LIKE THROUGH NDAR, FOR EXAMPLE, WHICH IS THE AUTISM NATIONAL DATABASE, HAS BEEN EXTREMELY DIFFICULT.

I WAS JUST GOING TO MENTION, AND I DON'T KNOW WHAT THE WORD "TOOLS" MEANT, BUT I THINK IN ADDITION TO SHARING DATA, SHARING PROTOCOLS AND ANALYTICAL PIPELINES, I THINK, REALLY NEEDS TO BE INCLUDED. AND THERE ARE VERY STRAIGHTFORWARD MECHANISMS THAT PEOPLE GENERALLY USE, BUT NOT EVERYBODY POSTS THEIR ANALYTICAL PIPELINE ON A GITHUB, FOR EXAMPLE, WHICH IS A FREE, OPEN ACCESS DATABASE. AND I THINK, THROUGH THESE STUDIES, THERE WILL DEFINITELY BE ANALYTICAL PIPELINES AND OTHER SORTS OF PROTOCOLS THAT WILL BE DEVELOPED AND SHOULD BE SHARED OPENLY.

DR. CANET-AVILES: CAN I SAY SOMETHING?

CHAIRMAN GOLDSTEIN: GO AHEAD, ROSA, PLEASE, YES.

DR. CANET-AVILES: THIS IS GREAT. AND I DON'T KNOW IF DR. LEVITT HAD A CHANCE TO ATTEND THE WORKSHOP LAST FRIDAY FOR THE DATA INFRASTRUCTURE, AND WE WERE TALKING ABOUT THE CONCEPT OF THE DATA BIOSPHERE WHICH COULD CONTAIN ANALYTICAL PIPELINES. THIS WOULD BE SOMETHING THAT WE WOULD DEFINITELY BE THINKING ABOUT IN PHASE II OF THIS IMPLEMENTATION. THAT'S AN EXCELLENT POINT. THANK YOU.

DR. LEVITT: YOU'RE WELCOME.

CHAIRMAN GOLDSTEIN: GREAT. ANY OTHER QUESTIONS OR DISCUSSION HERE? MARIA, DO WE HAVE A QUORUM SO WE CAN VOTE?

MS. BONNEVILLE: WE DO HAVE A QUORUM, BUT WE NEED A MOTION.

CHAIRMAN THOMAS: NEED A MOTION. SOMEBODY?

DR. YAMAMOTO: SO MOVED.

CHAIRMAN GOLDSTEIN: SECOND SOMEWHERE?

DR. LEVITT: SECOND.

CHAIRMAN GOLDSTEIN: OKAY. GREAT. LET'S SEE. ANY FURTHER DISCUSSION BEFORE WE CALL THE ROLL?

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MS. BONNEVILLE: I'M SORRY. WHO WAS THE SECOND? I WAS JUST TRYING TO FIND THE VOICE.

DR. LEVITT: IT WAS PAT.

MS. BONNEVILLE: THANK YOU, PAT. GREAT.

CHAIRMAN GOLDSTEIN: GREAT. MARIA BONNEVILLE.

MS. BONNEVILLE: CAN YOU CALL FOR PUBLIC COMMENT?

CHAIRMAN GOLDSTEIN: WE NEED PUBLIC COMMENT ON THIS ITEM. PUBLIC COMMENT PLEASE. DO WE HAVE ANY?

MS. BONNEVILLE: WE DO NOT HAVE ANY. YOU KNOW, WE FOLLOW THESE RULES.

CHAIRMAN GOLDSTEIN: KEVIN MARKS HAS SOMETHING TO SAY ALL OF A SUDDEN. KEVIN.

MR. MARKS: ALL OF A SUDDEN. SO WE'VE BEEN WORKING A LITTLE BIT ON THE BACKGROUND WITH RESPECT TO THE ISSUE OF THE CO-PI DEFINITION AND THE ALLOWANCE UNDER THE GAP. IN A QUICK REVIEW, OUR GAP DOES NOT ENVISION THE USE OF A CO-PI IN ANY OF THE ACTIVITIES. BUT WE'RE IN THE MIDST OF ACTUALLY REVIEWING OUR GAP AND PROPOSING CERTAIN REVISIONS. SO WE CAN ALWAYS TAKE THAT INTO CONSIDERATION. IF THIS COMMITTEE AND THE BOARD WISHES TO DO SO, WE CAN TAKE THAT INTO CONSIDERATION AS WE PROPOSE

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ADDITIONAL MODIFICATIONS.

CHAIRMAN GOLDSTEIN: SO I THINK THE IMPLICATION OF WHAT YOU JUST SAID IS WE CAN'T BY FIAT HAVE A PILOT PROGRAM ON THIS PARTICULAR CONCEPT THAT WE ARE DISCUSSING. AND THAT ANY CHANGE IN THE GAP WOULD HAVE TO COME THROUGH HERE AND THEN GO TO THE FULL BOARD BEFORE IT WAS IMPLEMENTED; IS THAT RIGHT?

MR. MARKS: IN A QUICK REVIEW OF THE GAP, YES. WE CAN EXPLORE A LITTLE BIT MORE WITH RELATION TO OUR FLEXIBILITY. AS IS STATED, THE CURRENT WORDING OF THE GAP DOES NOT ALLOW A CO-PI TYPE SITUATION.

CHAIRMAN GOLDSTEIN: OKAY. SO I GUESS WE CAN'T DO A PILOT PROGRAM ON THIS ONE. SO STRIKE THAT FROM THE PLAN. BUT IT SOUNDS LIKE WE SHOULD ASK KEVIN AND TEAM TO RESEARCH MAKING CHANGES IN THE GAP TO ALLOW SUCH A PILOT PROGRAM AND THEN BRING IT BACK TO THIS GROUP, I GUESS, AND THEN TO THE FULL BOARD, CORRECT?

MS. BONNEVILLE: CORRECT.

CHAIRMAN THOMAS: OKAY. ANYTHING ELSE BEFORE WE VOTE? OKAY. MARIA BONNEVILLE, NOW YOU CAN CALL THE ROLL.

MS. BONNEVILLE: HAIFA ABDULHAQ. DEBORAH

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DEAS.

DR. DEAS: YES.

MS. BONNEVILLE: MARK FISCHER-COLBRIE.

DR. FISCHER-COLBRIE: YES.

MS. BONNEVILLE: ELENA FLOWERS. JUDY GASSON.

DR. GASSON: YES.

MS. BONNEVILLE: LARRY GOLDSTEIN.

CHAIRMAN GOLDSTEIN: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: PAT LEVITT.

DR. LEVITT: YES.

MS. BONNEVILLE: DAVE MARTIN. SHLOMO MELMED.

DR. MELMED: YES.

MS. BONNEVILLE: CHRISTINE MIASKOWSKI.

DR. MIASKOWSKI: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: CARL WARE.

DR. WARE: YES.

MS. BONNEVILLE: KAROL WATSON. KEITH

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YAMAMOTO.

DR. YAMAMOTO: YES.

MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN GOLDSTEIN: OKAY. THANK YOU, EVERYBODY.

ON TO ITEM 4 IN THE AGENDA, ADDITIONAL AMENDMENTS TO CONCEPT PLANS FOR DISCOVERY, TRANSLATION, AND CLINICAL STAGE PROJECTS. LET'S TRY TO REMEMBER THE DISCUSSIONS WE JUST HAD SO WE DON'T HAVE TO REINVENT THE WHEEL WHEN WE TALK ABOUT THESE OTHER PROPOSED MODIFICATIONS. WHO'S GOT THIS?

DR. SAMBRANO: I'VE GOT THIS ONE. THANK YOU, MR. CHAIRMAN. LET ME SHARE MY SCREEN.

SO, AS YOU KNOW, WE HAVE ONGOING OPPORTUNITIES TO FUND DISCOVERY, TRANSLATIONAL, AND CLINICAL STAGE PROGRAMS THAT HAVE BEEN RUNNING NOW SINCE ABOUT 2015. AND SO WE PERIODICALLY COME TO YOU WITH SOME TWEAKS OR CHANGES FOR THESE CONCEPTS. SO WE ARE COMING TO YOU AGAIN WITH SOME PROPOSED CHANGES, WHICH I WILL GO OVER.

SO I DIVIDED THIS UP TO KIND OF FOCUS IN ON CHANGES TO SPECIFIC CONCEPTS. AND THEN AT THE END I'M GOING TO TALK ABOUT SOME OF THE GLOBAL ONES THAT APPLY TO ALL OF THESE CONCEPTS. AND WE PROVIDED THE DOCUMENT. SO YOU WILL SEE THE ACTUAL

CONCEPT DOCUMENT WITH TRACK CHANGES WHERE YOU CAN SEE HOW THOSE ARE BEING IMPLEMENTED. THE SLIDES ARE JUST GOING TO PRESENT A HIGH LEVEL OVERVIEW OF THESE.

SO FOR THE DISCOVERY 2 CONCEPT, DISC2 IS OUR QUEST PROGRAM, WHICH SEEKS TO SUPPORT WORK TO IDENTIFY A PRODUCT CANDIDATE OF SOME KIND, IN MANY CASES OR PROBABLY IN MOST CASES, A THERAPEUTIC OF SOME SORT, BUT IN OTHER CASES IT MAY BE A TOOL.

SO ONE OF THE PROPOSALS THAT WE HAVE IN THIS CONCEPT IS TO INCREASE THE DURATION ALLOWABLE FOR THERAPEUTIC CANDIDATE AWARDS FROM TWO YEARS TO THREE YEARS. AND THIS IS BASED, IN PART, ON FEEDBACK AND JUST OUR OBSERVATION OF APPLICANTS AND AWARDEES IN GETTING TO THAT END GOAL OF IDENTIFYING A THERAPEUTIC CANDIDATE AND FEELING THAT, BY ALLOWING MORE TIME, YOU'RE LIKELY TO INCREASE THE SUCCESS OF THEM ACCOMPLISHING THAT GOAL. SO THAT IS ONE OF THE PROPOSALS.

AND ALIGNED WITH THAT IS AN INCREASE IN THE BUDGET TO ACCOUNT FOR THE INCREASE IN TIME FROM 24 TO 36 MONTHS, BUT ALSO TO ALLOW A SMALL INCREASE IN ADJUSTMENT TO TOTAL PROJECT COSTS THAT ACCOUNT FOR INCREASES IN CAPS TO PERSONNEL AS WELL AS JUST THE ADJUSTMENT OF GOODS AND SERVICES AND INFLATION

OVERALL.

ONE OF THE ADDITIONAL ELEMENTS TO THIS IS THE OPPORTUNITY TO ADD UP TO 200,000 OVER THAT CAP THAT APPLICANTS MAY REQUEST WITH JUSTIFICATION FOR SPECIFIC ACTIVITIES. AND THESE SPECIFIC ACTIVITIES ARE OBTAINING AND/OR SHARING DEVELOPMENT COMPATIBLE LINES, TESTING MULTIPLE LINES TO ENSURE QUALITY OF THEIR SELECTED DEVELOPMENT CANDIDATE, AND TO ADDRESS SCIENTIFIC DIVERSITY.

SO AN EXAMPLE THAT HAS COME UP OFTEN, PARTICULARLY SINCE OUR COVID INITIATIVES, WAS INCREASING THE NUMBER OF LINES THAT ARE TESTED TO ENSURE THAT THERE IS RACIAL AND ETHNIC DIVERSITY REPRESENTED IN THOSE LINES THAT ARE TESTED. SO BY PROVIDING THIS ALLOWANCE, HOPEFULLY IT MAY INCENTIVIZE GREATER STUDY OF THOSE DIVERSE LINES AS WELL AS THE OPPORTUNITY TO MAKE THAT A REALITY FOR THOSE. SO THAT'S JUST THE DISC2.

WE HAVE ONE CHANGE THAT APPLIES ACROSS DISC2 AND THEN OUR TRANSLATIONAL CONCEPT, WHICH IS TO INCREASE THE ALLOWANCE TO AWARD LAUNCH FROM 60 DAYS CURRENTLY TO 90 DAYS. WE'VE BEEN GOING BACK AND FORTH A LITTLE BIT. WE TRIED TO BE VERY PROGRESSIVE IN THIS, AND WE WENT DOWN TO AS LITTLE AS 30 DAYS WHEN WE LAUNCHED THE COVID PROGRAM. I

THINK THERE WAS REALLY AN EFFORT TO SPEED UP WHAT WE DO AS MUCH WE COULD. BUT THAT WAS NOT SUSTAINABLE BEYOND THAT, AND WE FOUND THAT 90 DAYS IS A MORE COMFORTABLE WINDOW THAT ALLOWS US TO WORK WITH THE APPLICANTS OR AWARDEES TO BE IN ORDER TO NEGOTIATE THE FINAL CONTRACT AND LAUNCH THOSE AWARDS. SO THAT APPLIES TO THOSE TWO.

FOR THE CLINICAL PROGRAM, SO THE CLIN 2 IS THE CONCEPT AND OPPORTUNITY THAT FUNDS CLINICAL TRIAL PROJECTS. AND SO THE MAIN CHANGE HERE IS A PROPOSAL TO CHANGE THE BASIS FOR DETERMINING WHAT THE AWARD MAXIMUM AND COFUNDING AMOUNTS WOULD BE.

SO CURRENTLY THE WAY WE DO THIS IS BASED ON WHETHER A PROJECT IS A PHASE I, PHASE II, OR PHASE III CLINICAL TRIAL. WE HAVE FOUND OVER TIME THAT NOT ALL PROJECTS FIT VERY NEATLY WITHIN THE I, II, OR III PHASE DESIGNATION. SOMETIMES THERE'S I-II'S OR PROJECTS THAT MAYBE SHOULD BE LABELED A I, BUT ARE NOT, OR A II AND VICE VERSA. AND ALSO THERE IS THE DESIRE FROM SOME APPLICANTS TO TRY TO LABEL AND FIT THEIR PROJECT WHERE THEY CAN TO ACCOUNT FOR THE AWARD MAXIMUM THAT THEY CAN CLAIM OR FOR THE COFUNDING ALLOWANCE.

SO WE THOUGHT WE WOULD SIMPLIFY IT A BIT. INSTEAD OF USING THE PHASE I, II, AND III, PROPOSE

THAT STUDIES THAT ARE FIRST IN HUMAN, THAT ARE TESTING A THERAPEUTIC CANDIDATE IN A GIVEN DISEASE INDICATION WITH A GIVEN ROUTE OF ADMINISTRATION WOULD THEN HAVE AN AWARD MAX OF 12 MILLION FOR NONPROFIT, 8 MILLION FOR FOR-PROFIT; COFUNDING, NONE FOR NONPROFIT AND 30 PERCENT FOR FOR-PROFIT. AND THOSE ARE IDENTICAL TO WHAT WE CURRENTLY HAVE FOR OUR PHASE I STUDIES. SO THAT ALIGNS PRETTY WELL, BUT WE'RE JUST NOW CALLING IT FIRST IN HUMAN.

ALL SUCCEEDING STUDIES, SO ANYTHING AFTER FIRST IN HUMAN, WOULD THEN ENTER INTO THAT SECOND TIER WHERE THEY CAN REQUEST UP TO 15 MILLION, WHETHER NONPROFIT OR FOR-PROFIT, AND WOULD REQUIRE A COFUNDING OF 40 PERCENT, AGAIN, WHETHER FOR-PROFIT OR NONPROFIT. AND THIS IS ALIGNED WITH WHAT WE CURRENTLY HAVE FOR PHASE II STUDIES COMING IN. SO THAT'S THE PROPOSAL FOR CLIN2.

FOR CLIN1 THIS IS JUST A VERY SIMPLE CHANGE ACTUALLY OF INCLUDING LANGUAGE FOR THOSE THAT ARE PROPOSING ALLOGENEIC CELL THERAPIES. THIS IS JUST ENSURING THAT THEY MEET THE DONOR ELIGIBILITY REQUIREMENTS, WHICH WE ALREADY HAVE IN THE DISCOVERY AND TRAN CONCEPTS. SO WE ARE JUST BRINGING IT INTO CLIN1 JUST TO MAKE SURE THAT THAT IS CONSISTENT.

AND IN TERMS OF GLOBAL CHANGES THAT APPLY

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TO ALL THREE, DISCOVERY, TRAN, AND CLIN, WE TALKED ABOUT THE DATA SHARING PLAN REQUIREMENTS. SO HERE AGAIN, JUST TO EMPHASIZE WHAT WE ARE DOING IS SIMPLY MAKING A CHANGE IN THE LANGUAGE. WE ACTUALLY HAVE ALREADY HAD LANGUAGE IN THESE CONCEPTS THAT BEGAN AROUND THE TIME THAT WE INSTITUTED THE COVID OPPORTUNITIES FOR HAVING A DATA SHARING PLAN. AND SO WE HAVE SINCE THAT TIME GOTTEN TO SEE A LITTLE BIT ABOUT WHAT FOLKS DO WITH A PLAN. SOME ARE NOT SO GREAT; SOME ARE GREAT. SO WE ARE HOPING TO TWEAK THE LANGUAGE A LITTLE BIT AND SAY THAT NOW, INSTEAD OF JUST EXPECTING, THAT WE REQUIRE AWARDEES TO DEVELOP AND EXECUTE A DATA SHARING PLAN. WE ALSO REQUIRE THAT THEY SHARE, IN ACCORDANCE TO FAIR DATA PRINCIPLES, AS WELL AS ALSO TO ALLOCATE FUNDS IN THE PROPOSED BUDGET TO ALLOW FOR EITHER PERSONNEL AND/OR ACTIVITIES THAT RELATE TO THE DATA SHARING. LET'S SEE.

WE ARE UPDATING DEI LANGUAGE. THESE ARE NOT SIGNIFICANT UPDATES IN THE LANGUAGE BECAUSE THAT ALSO HAS EXISTED IN ALL OF THESE CONCEPTS FOR A WHILE, BUT WE'RE TRYING TO REFLECT SOME OF THE IMPROVEMENTS THAT WE CONTINUE TO MAKE OR CONSOLIDATING OUR DEI SECTIONS TO MAKE IT CLEARER FOR APPLICANTS AND FOR US TO PROVIDE BETTER

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INSTRUCTION AS WELL AS TO REFLECT THE BOARD FEEDBACK THAT WE HAVE RECEIVED IN THIS ARENA.

WE ARE ALSO JUST BROADLY REMOVING EXPIRED REFERENCES TO THE CIRM 2.0, STREAMLINING THE DOCUMENT. AND SO YOU MAY HAVE NOTICED THAT THE TEMPLATES UPON WHICH WE HAVE THEM ARE ALL DIFFERENT AND NEW, BUT THEY'RE ALL CONSISTENT WITH ONE ANOTHER, AND HOPEFULLY THIS WILL HELP PRESENT THE INFORMATION IN A CLEARER FASHION FOR EVERYONE TO SEE.

AND SO SPEAKING OF CONSISTENCY, ONE OF THE OTHER EXAMPLES IN WHICH WE ARE TWEAKING LANGUAGE TO ENSURE THAT THIS IS THE CASE IS IN THE ELIGIBILITY OF THE CANDIDATES THAT CAN COME IN FOR EACH OF THESE OPPORTUNITIES. ONE OF THE EXAMPLES THAT WE ARE ALIGNING IS IN THE FIRST BULLET WHERE IN THE TRANSLATION PROGRAM, FOR A FEW HISTORICAL REASONS, WE WERE NOT ALLOWING MINIMALLY MANIPULATED BONE MARROW, CORD BLOOD, OR UNMODIFIED HSC'S IN THAT PARTICULAR OPPORTUNITY, BUT IT WAS BEING ALLOWED IN DISCOVERY AND CLINICAL. SO JUST TO MAKE IT CONSISTENT, WE'RE GOING TO ALLOW IT IN TRAN SO THAT THEY ARE ALL BASICALLY THE SAME, AND THERE WILL BE LESS CONFUSION, I THINK, AMONG APPLICANTS OF WHAT QUALIFIES OR WHAT DOES NOT.

AND THEN I THINK WITH THAT -- SO THAT KIND OF TAKES CARE OF THE PROPOSED CHANGES, BUT WE ALSO DO HAVE A QUESTION THAT MAY GO INTO A LITTLE BIT OF DISCUSSION, WHETHER NOW THAT WE HAVE MOVED FORWARD WITH THE DISCOVERY 0 CONCEPT THAT HAS A GENETIC RESEARCH DEFINITION WHICH IS A LITTLE BIT DIFFERENT THAN THE GENE THERAPY DEFINITION THAT WE CURRENTLY USE, JUST GOING TO GO TO THE NEXT SLIDE SO YOU CAN SEE THEM SIDE BY SIDE, CURRENTLY WE HAVE BEEN USING A GENE THERAPY DEFINITION SHOWN ON THE LEFT THAT WAS ADOPTED BY THE BOARD A FEW YEARS AGO, THIS WAS EVEN BEFORE PROP 14, IN ORDER TO INCLUDE THIS TYPE OF RESEARCH TO BE FUNDED. AND CURRENTLY WE FUND IT WITHOUT ANY NEED FOR THERE TO BE A STEM CELL OR PROGENITOR CELL COMPONENT. THIS IS JUST STRAIGHT UP GENE THERAPY. AND SO WE DEFINE IT AS SHOWN ON THE LEFT.

NOW, THE DISC-0 GENETIC RESEARCH DEFINITION THAT IS IN THE DISC-0 CONCEPT IS A BIT BROADER. IT'S INCLUSIVE OF WHAT'S UNDER THE GENE THERAPY, BUT IT EXTENDS IT TO ALLOW FOR ELEMENTS SUCH AS INTRODUCING ANY NUCLEIC ACID INTO A CELL LIKE AN MRNA OR AN ANTISENSE OLIGO INTO CELLS. AND SO I THINK THE QUESTION FOR DISCUSSION IS NOT, JUST TO EMPHASIZE, THIS IS NOT CURRENTLY IN THE CONCEPT

DOCUMENTS, IS THE QUESTION OF WHETHER THAT SHOULD BE BROADENED TO BE LIKE IT IS IN DISC-0 OR WHETHER IT SHOULD REMAIN A LITTLE TIGHTER AS IS SHOWN ON THE LEFT AND HOW WE'VE BEEN USING IT RECENTLY.

SO THAT CONCLUDES MY PRESENTATION, AND HAPPY TO ADDRESS ANY QUESTIONS THERE MAY BE.

CHAIRMAN GOLDSTEIN: LET'S DO THIS, GUYS. I DO HAVE ONE GENERAL QUESTION FOR GIL. THEN I THINK WE SHOULD PROBABLY ORGANIZE THE DISCUSSION SO THAT FOR EACH TYPE OF GRANT WE DISCUSS BRIEFLY THE PROPOSED CHANGES. I THINK IF WE TRY TO DISCUSS ALL OF THOSE PROPOSED CHANGES AT ONCE, WE'RE GOING TO GET A LITTLE CONFUSED, AND IT WILL BE HARD TO MANAGE THE DISCUSSION.

SO THE GENERAL QUESTION TO GIL IS WE JUST HAD A LONG DISCUSSION WITH ROSA AND MARIA MILLAN ABOUT DATA SHARING REQUIREMENTS AND HELPFUL LANGUAGE THAT WILL BE ADDED TO THE GUIDANCE DOCUMENTS. IS THAT SAME APPROACH GOING TO BE USED FOR THE CLINS, TRAN, AND OTHER DISCS?

DR. SAMBRANO: YES. SO OUR APPROACH FOR ALL OF THIS IS TO THINK ABOUT DATA SHARING IN A MORE HOLISTIC WAY AS IT APPLIES TO ALL OF OUR PROGRAMS. SO NOT REALLY RESERVED JUST FOR ONE. SO ABSOLUTELY, YES.

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CHAIRMAN GOLDSTEIN: AND THE HELPFUL RESOURCES FOR APPLICANTS. GREAT.

DISCUSSION OR QUESTIONS ABOUT THE CONCEPT PLAN CHANGES FOR DISC2 PLEASE. ANYBODY GOT ANYTHING THEY WANT TO KNOW OR CONTRIBUTE? GOING ONCE. GOING TWICE. WE ARE OUT OF THERE.

TRAN? NO. YOU GUYS HAVE OBVIOUSLY DONE A GREAT JOB DRAFTING THESE CHANGES.

CLIN1 CHANGES, QUESTIONS, COMMENTS? NOPE.

CLIN2? OKAY.

THEN FINALLY, GIL'S QUESTION ABOUT THE DEFINITION OF GENE THERAPY AND GENETIC RESEARCH. I DID HAVE ONE SUGGESTION. I KNOW IN OTHER PLACES WE HAVE SAID "AS THEY APPLY TO STEM CELLS AND REGENERATIVE MEDICINE" AS A WAY OF AT LEAST NARROWING THE SCOPE A LITTLE BIT. IS THAT SOMETHING WE CAN DO HERE ALSO, GIL?

DR. SAMBRANO: YES. AND I THINK THAT'S ALSO BEEN TRUE OF THE GENE THERAPY DEFINITION THAT WE CURRENTLY HAVE. I THINK THERE WAS A STRONG FEELING FROM THE BOARD THAT IT SHOULD BE REGENERATIVE IN SOME WAY. SO WE HAVE THE SAME DEFINITION OF REGENERATIVE MEDICINE THAT WE WOULD APPLY TO THIS.

CHAIRMAN GOLDSTEIN: OKAY. GREAT. THANK

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YOU. OTHER -- J.T.

CHAIRMAN THOMAS: SO, GIL, I THINK IN THE SPIRIT OF INCORPORATING WHAT'S IN PROP 14, THAT EXPANDING THE SCOPE BEYOND THE ORIGINAL LANGUAGE THAT WE'VE BEEN USING FOR GENE THERAPY TO INCORPORATE THE BROADER GENETIC IDEA MAKES EMINENT SENSE. SO I THINK WE NEED TO GIVE THAT VERY SERIOUS CONSIDERATION.

DR. YAMAMOTO: GIL, COULD YOU PUT THAT LANGUAGE BACK UP AGAIN?

CHAIRMAN GOLDSTEIN: GREAT IDEA. GOOD. PLEASE.

DR. SAMBRANO: GIVE ME A SECOND.

CHAIRMAN GOLDSTEIN: J.T., YOU'VE GOT YOUR HAND UP AGAIN.

CHAIRMAN THOMAS: THAT'S THE SAME HAND. I WAS MUTED. I'M SURE MARIA MAY HAVE DONE THAT TO ME INTENTIONALLY.

MS. BONNEVILLE: NOT THIS TIME.

CHAIRMAN THOMAS: AS THE TECHNOLOGY MARCHES INEXORABLY ALONG AND MORE THINGS ARE APPLICABLE TO THE REGENERATIVE MEDICINE SPACE, I THINK WE NEED TO KEEP PACE OF THAT. AND I THINK THIS DISC-0 LANGUAGE HERE IS REFLECTIVE THAT THOSE NEW TECHNOLOGIES ARE BECOMING MORE AND MORE

APPLICABLE. SO I WOULD BE IN FAVOR OF ENLARGING THE SCOPE TO ALLOW FOR THIS TERMINOLOGY.

CHAIRMAN GOLDSTEIN: OTHER THOUGHTS, COMMENTS ON THIS? I'LL JUST ADD THAT I WAS AT A SEMINAR BEFORE THIS ONE THAT ACTUALLY WAS USING DIFFERENT WAYS OF TRANSFORMING CELL FATE, FOR EXAMPLE, FROM ASTROCYTES TO NEURONS, WHICH COULD BE A VERY POWERFUL APPROACH. I THINK THIS WOULD ALLOW THOSE SORTS OF APPROACHES TO BE INCLUDED WHICH IS GOOD IN MY MIND.

DR. CREASEY: LARRY, WOULD YOU LIKE ME TO SHARE THE TWO SLIDES WE HAVE THAT ARE IN THIS TOPIC?

CHAIRMAN GOLDSTEIN: SURE.

DR. CREASEY: SO MY COLLEAGUES AND I PUT OUR HEADS TOGETHER AND THOUGHT WHAT WOULD BE THE RATIONALE FOR EXPANDING THE SCOPE OF GENE THERAPY FOR CIRM FUNDING OPPORTUNITIES FOR ALL OUR PILLARS. AS J.T. JUST POINTED OUT, PROPOSITION 14 INDICATES THE FUNDING OF GENETIC RESEARCH, WHICH, AGAIN, YOU SAW THE DEFINITION. AND JUST TO REMIND EVERYONE, ESPECIALLY IN THE LAST TWO YEARS, THERE HAS BEEN SIGNIFICANT PROGRESS IN THE AREA OF RNA SCIENCE THAT APPLIES TO VACCINES AND THERAPEUTICS, INCLUDING REGENERATIVE MEDICINE. AGAIN, THERE WAS A TESTIMONIAL FROM LARRY RIGHT NOW.

SO CIRM HAS THE UNIQUE OPPORTUNITY IN THE CREATION OF NEW SOLUTIONS TO ACCELERATE APPLICATION OF GENE THERAPIES FOR UNMET MEDICAL NEEDS. AND IF YOU'VE BEEN FOLLOWING THE LITERATURE, ESPECIALLY IN THE LAST COUPLE YEARS AND THE LAST EVEN SINCE OCTOBER, THERE HAVE BEEN DEVELOPMENT OF NEW TECHNOLOGIES THAT WILL ALLOW FOR NOVEL AND SUCCESSFUL APPLICATION OF THESE MODALITIES TO AREAS WHERE WE REALLY ARE DESPERATELY LOOKING FOR SOLUTIONS, SUCH AS RNA VACCINE FOR NEURODEGENERATIVE DISEASES.

SO I WILL SHOW YOU IN THE NEXT SLIDE THAT THESE APPROACHES HAVE BEEN DEVELOPED AND APPLIED FOR RARE AND UNMET MEDICAL NEEDS, INCLUDING THE ASO'S, SRNA, AND, AS YOU KNOW, MOST RECENTLY MESSENGER RNA. AND WE ALL HAVE HAD HOPEFULLY THE VACCINE AND ALREADY APPRECIATE THE IMPORTANCE OF M-RNA IN RESOLVING SOME OF OUR DISEASES.

WHAT'S SO EXCITING IS THAT THE M-RNA RESEARCH HAS EXPLODED TO INCLUDE MULTIPLE APPLICATIONS THAT ARE REALLY IMPORTANT TO US, SUCH AS THE AREA OF MULTIPLE SCLEROSIS AS AN AUTOIMMUNE DISEASE, NOT AS AN INFECTIOUS DISEASE, BUT AS AN AUTOIMMUNE DISEASE; BONE REGENERATION; AUTOIMMUNE DISEASES. MODERNA IS INVESTING IN AUTOIMMUNE

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DISEASE BY SHOWING JUST IN ANIMAL MODELS THAT CAR-T CELLS ARE INCREASED AS WELL AS TREG'S ARE DEVELOPED IN THE PRESENCE OF THE SPECIFIC M-RNA'S THAT THEY ARE USING IN AUTOIMMUNE DISEASES.

MOST RECENTLY MODERNA AND PENN STATE HAVE SHOWN, ALSO IN PRECLINICAL STUDIES, THAT THEY CAN THROUGH MESSENGER RNA TECHNOLOGY RESOLVE SOME CARDIAC DISEASE, AGAIN IN ANIMAL MODELS. ALL THIS IS A TESTAMENT TO THE FACT THAT, IN ORDER FOR CIRM TO MAINTAIN IT'S BEING ON THE FOREFRONT AND ACCELERATING SCIENCE, I THINK IT MAKES SENSE FOR US TO BE INVOLVED IN THAT RESEARCH AND ALLOW US TO ASSIST THOSE GRANTEES WHO ARE INTERESTED IN THIS AREA AND IN ACCELERATING THE WORK. AND WHO KNOWS, IT MAY TURN OUT TO BE MANY OTHER DISEASES WE'VE BEEN WANTING SOLUTIONS FOR MAY COME OUT FROM THIS TECHNOLOGY. AND SO FOR THAT, I THINK I PERSONALLY WOULD LIKE THE BOARD TO CONSIDER THIS TECHNOLOGY TO BE PART OF ARMAMENTARIUM MOVING FORWARD.

CHAIRMAN GOLDSTEIN: GREAT. THANK YOU, ABLA.

ANY OTHER DISCUSSION BEFORE -- WELL, I GUESS I HAVE TO ANNOUNCE. MARIA BONNEVILLE JUST LET ME KNOW THAT WE SEEM TO HAVE LOST QUORUM. SO WE ARE NOT GOING TO BE ABLE TO DO ANYTHING FINAL TODAY, I

GATHER, BUT WE CAN GET A SENSE OF THE COMMITTEE. BUT THEN HOW DO WE BRING THIS TO A FINAL APPROVAL BEFORE IT GOES TO THE BOARD, MARIA?

MS. BONNEVILLE: YOU WOULD JUST MENTION THAT THE SENSE OF THE COMMITTEE WAS THAT THEY WERE IN FAVOR OF IT, ALTHOUGH NOT ALL MEMBERS WERE PRESENT AND YOU DID NOT HAVE A QUORUM, AND THEN THE FINAL VOTE WOULD BE TAKE PLACE AT THE BOARD MEETING.

CHAIRMAN GOLDSTEIN: OKAY. GREAT. SO WE CAN GO TO THE BOARD. GOOD.

SO ANY FINAL COMMENTS OR QUESTIONS ABOUT THE DEFINITION CHANGES? MARIA MILLAN.

DR. MILLAN: IF THE SUBCOMMITTEE IS IN FAVOR OF EXPANSION OF THE DEFINITION, WOULD YOU LIKE US TO BRING THAT FOR BOARD CONSIDERATION AT THE MARCH 24TH MEETING?

CHAIRMAN GOLDSTEIN: I THINK THAT WOULD BE SENSIBLE. IT'S A SIGNIFICANT CHANGE. IT'S A VERY POSITIVE CHANGE, I THINK, IN TERMS OF TECHNOLOGY. I DO THINK WE'RE GOING TO STILL WANT TO KEEP A FOCUS ON STEM CELL AND REGENERATIVE MEDICINE APPROACHES AS MUCH AS POSSIBLE. YES, I THINK THAT SHOULD COME TO THE BOARD.

DR. DEAS: I AGREE. THIS REALLY EXPANDS THE PORTFOLIO BECAUSE WITH RNA THERAPEUTICS, THERE'S

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A LOT OF NEW DEVELOPMENTS.

CHAIRMAN GOLDSTEIN: OTHER COMMENTS OR QUESTIONS? OKAY. SO, MARIA BONNEVILLE CAN WE VOTE ON THESE IN A BATCH, OR DO WE HAVE TO GO THROUGH ONE BY ONE?

MS. BONNEVILLE: YOU CAN VOTE IN A BATCH.

CHAIRMAN GOLDSTEIN: GREAT. SOMEBODY WANT TO MOVE FOR APPROVAL?

DR. HIGGINS: I WILL MOVE FOR APPROVAL.

DR. DEAS: SECOND.

CHAIRMAN GOLDSTEIN: THANK YOU.

MR. TORRES: I THOUGHT WE COULDN'T BECAUSE WE DON'T HAVE A QUORUM.

MS. BONNEVILLE: IT'S NOT A FORMAL VOTE. IT'S JUST A SENSE OF THE COMMITTEE.

MR. TORRES: SO A CONSENSUS OF THE COMMITTEE. OKAY.

CHAIRMAN GOLDSTEIN: PUBLIC COMMENT AT THIS POINT, CORRECT?

MS. BONNEVILLE: YES.

CHAIRMAN GOLDSTEIN: IS THERE ANY PUBLIC COMMENT?

MS. BONNEVILLE: KEVIN, GO AHEAD.

MR. MARKS: BEFORE WE DO, I JUST WANT TO MAKE SURE THAT WE -- SO FROM A PERSONAL PERSPECTIVE,

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I LOOK AT THIS AS TWO DIFFERENT SITUATIONS. SO YOU BATCH THE CONCEPTS, AND THEN THERE WAS A SEPARATE CONSIDERATION OF THE EXPANDED DEFINITION OF GENETIC RESEARCH. I JUST WANT TO MAKE CLEAR ON WHAT THE RECOMMENDATIONS ARE SO WHEN WE POSITION THIS FOR THE BOARD MEETING, WE CAN CLEARLY ARTICULATE IT.

SO I'M FINE IF WE WANT TO DO IT ALTOGETHER, JUST THE APPROVAL OF EVERYTHING, OR DO YOU WANT TO BATCH IT UP WITH THE CONCEPT CONSIDERATIONS AND APPROVALS AND THEN THE APPROVAL OF THE EXPANDED DEFINITION?

MS. BONNEVILLE: ISN'T THE DEFINITION PART OF THE CONCEPT CHANGE?

CHAIRMAN GOLDSTEIN: YEAH, I BELIEVE IT IS. KEVIN, I THINK THAT'S A GOOD QUESTION. I PERSONALLY WOULD SAY WE OUGHT TO JUST DO THEM AS ONE BATCH. I CAN TELL FROM THE DISCUSSION --

MR. TORRES: HERE. HERE.

CHAIRMAN GOLDSTEIN: -- EVERYBODY IS GOING TO BE IN FAVOR OF EVERYTHING THAT'S BEEN PROPOSED. THERE'S BEEN INSIGHTFUL DISCUSSION AND BACK AND FORTH. I THINK WE ARE IN GOOD SHAPE TO DO THAT.

MR. MARKS: THANK YOU.

CHAIRMAN GOLDSTEIN: DOES ANYBODY OBJECT TO THAT APPROACH? GREAT. OKAY.

PUBLIC COMMENT. STILL NONE, RIGHT?

MS. BONNEVILLE: NOT THAT I CAN FIND.

CHAIRMAN THOMAS: LET'S VOTE ON A SENSE OF THE COMMITTEE.

MS. BONNEVILLE: HAIFA. DEBORAH DEAS.

DR. DEAS: YES.

MS. BONNEVILLE: MARK FISCHER-COLBRIE.

DR. FISCHER-COLBRIE: YES.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: YES.

MS. BONNEVILLE: LARRY GOLDSTEIN.

CHAIRMAN GOLDSTEIN: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: PAT LEVITT.

DR. LEVITT: YES.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: YES.

MS. BONNEVILLE: CHRISTINE MIASKOWSKI.

DR. MIASKOWSKI: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: KEITH YAMAMOTO.

DR. YAMAMOTO: YES.

MS. BONNEVILLE: THANK YOU.

CHAIRMAN GOLDSTEIN: OKAY. THE SENSE OF THE COMMITTEE HAS PASSED.

LET'S SEE. ACCORDING TO MY AGENDA, THE LAST THING IS ANY ADDITIONAL PUBLIC COMMENT. IT SOUNDS LIKE DO WE HAVE ANYBODY WHO WANTS TO MAKE A GENERAL OR OTHER COMMENT? NO. I THINK WE'VE BEEN VERY EFFICIENT, GUYS.

MR. TORRES: THANK YOU.

MS. BONNEVILLE: THANK YOU, EVERYONE. SEE YOU AT THE BOARD MEETING MARCH 24TH.

MR. TORRES: THANKS FOR THE WARNING.

(THE MEETING WAS THEN CONCLUDED AT 2:14 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 VIRTUAL PROCEEDINGS BEFORE THE SCIENCE SUBCOMMITTEE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON MARCH 7, 2022, 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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