BEFORE THE
TREATMENT AND CURES ACCESSIBILITY AND AFFORDABILITY
WORKING GROUP
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: MAY 14, 2024
1 P.M.

REPORTER: BETH C. DRAIN, CA CSR
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I N D E X

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MAY 14, 2024; 1 P.M.

CHAIRPERSON BONNEVILLE: HI, EVERYONE. THANK YOU FOR JOINING US TODAY FOR THE ACCESS AND AFFORDABILITY WORKING GROUP MEETING. I'D LIKE TO CALL THE MEETING TO ORDER. GEOFF, CAN YOU CALL THE ROLL PLEASE.

DR. LOMAX: YEAH. KIM BARRETT. DAN BERNAL. MARIA BONNEVILLE.

CHAIRPERSON BONNEVILLE: PRESENT.

DR. LOMAX: ANN BOYNTON. JAMES DE BENEDETTI.

MR. DE BENEDETTI: HERE.

DR. LOMAX: DANA DORNSIFE. TED GOLDSTEIN.

DR. GOLDSTEIN: HERE.

DR. LOMAX: DAVID HIGGINS.

DR. HIGGINS: PRESENT.

DR. LOMAX: DARIUS LAKDAWALLA. HARLAN LEVINE. PAT LEVITT. ADRIANA PADILLA.

DR. PADILLA: HERE.

DR. LOMAX: AMMAR QADAN. MAHESWARI SENTHIL. ADRIENNE SHAPIRO.

MS. SHAPIRO: HERE.

DR. LOMAX: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: HERE.

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CHAIRPERSON BONNEVILLE: THANK YOU, GEOFF.

JUST AS A QUICK POINT OF ORDER, WE'RE GOING TO MOVE THE AGENDA ITEMS AROUND A BIT. WE'RE GOING TO START WITH THE UPDATE FROM ROSA ON THE STRATEGIC ALLOCATION FRAMEWORK. WE'LL THEN MOVE INTO CLOSED SESSION FOR AN UPDATE ON SOME OF OUR CLIN PORTFOLIO AND BLA STATUS. AND THEN WE'RE GOING TO COME OUT OF CLOSED SESSION, AND THEN WE'LL TAKE UP COMMUNITY CARE CENTERS OF EXCELLENCE, THE UPDATE ON THE RFA, AND THE PATIENT SUPPORT PROGRAM UPDATE. SO I'M GOING TO TURN IT OVER ROSA NOW TO GIVE THE PRESENTATION. SO THANK YOU, ROSA.

DR. CANET-AVILES: THANK YOU, MADAM VICE CHAIR, MEMBERS OF THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP. AND THANK YOU, EMILY, FOR SHARING THE SLIDES. APPRECIATE THAT.

SO TODAY WE ARE GOING TO PROVIDE AN UPDATE ON THE STRATEGIC ALLOCATION FRAMEWORK. AS MANY OF YOU KNOW, THE CIRM STAFF PRESENTED BACK AT THE MARCH ICOC MEETING A PLAN TO DEVELOP A STRATEGIC ALLOCATION FOR THE REMAINING FUNDS OF CIRM. AND WE'RE GOING TO UPDATE YOU ON THAT PLAN AND THE PROCESS AND THE NEXT STEPS ON HOW THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP CAN HELP US MOVE THINGS FORWARD.

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SO THE GOALS FOR TODAY IS TO PROVIDE A BACKGROUND ON THIS PRIORITIZATION TO THE ACCESS AND AFFORDABILITY WORKING GROUP. ASSUMING THAT MANY OF YOU ARE ALREADY FAMILIAR WITH IT, WE ARE GOING TO DO THIS AT A HIGH LEVEL. AS A REMINDER, MANY OF THE BACKGROUND MATERIALS AND THE STRATEGIC ALLOCATION FRAMEWORK DOCUMENT ARE PROVIDED AS BACKGROUND DOCUMENTS FROM THE ICOC MEETING IN MARCH. AND IF ANYBODY WANTS ACCESS, PLEASE JUST ASK US.

AND WE WILL INTRODUCE GOAL NO. 2 OF THE FOUR GOALS OF THE STRATEGIC ALLOCATION FRAMEWORK PLAN. AND THEN WE WILL DISCUSS HOW THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP CAN HELP US, TOGETHER WITH THE NEURO TASK FORCE AND THE SCIENCE SUBCOMMITTEE, CAN HELP US MOVE FORWARD WITH THE RECOMMENDATIONS FOR THE SEPTEMBER DEADLINE THAT WE HAVE.

WITH THAT, THE PRESENTATION OF REVIEW IS DIVIDED IN THREE POINTS. BACKGROUND, I'LL JUST GO OVER AGAIN THE STRATEGIC ALLOCATION FRAMEWORK AND THE PROCESS THAT WE HAVE DEVELOPED TO PROVIDE RECOMMENDATIONS TO THE BOARD. I'LL THEN DIG DEEPLY INTO GOAL 2 AND WHAT THE INPUT FROM THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP IS WITH REGARDS TO THE QUESTIONS THAT WE HAVE DEVELOPED

TO FULFILL THIS GOAL, THE RECOMMENDATIONS. AND THEN WE WILL GO OVER A TIMELINE SO THAT WE ARE ALL IN THE SAME PAGE AS TO WHEN ARE THINGS DUE AND WHEN WE WILL BE MEETING AGAIN TO DISCUSS THOSE RECOMMENDATIONS WITH THIS GROUP.

SO WITHOUT FURTHER -- NEXT SLIDE. SORRY. I WILL MAKE SURE THAT I SAY NEXT. MY APOLOGIES.

SO ALL OF THIS IS, AS ALWAYS, WITHIN THE FRAMEWORK OF OUR FIVE-YEAR STRATEGIC PLAN. THAT'S HOW WE DEVELOP OUR STRATEGIC ALLOCATION FRAMEWORK. AND THE STRATEGIC ALLOCATION FRAMEWORK THAT WE HAVE PRESENTED IS BASICALLY A STRUCTURED AND DATA-DRIVEN APPROACH THAT WILL ALLOW US TO PRIORITIZE RESOURCE ALLOCATION AND PROVIDE FURTHER GRANULARITY IN TERMS OF IMPACT GOALS AND THEIR SUCCESS MEASURES THAT ULTIMATELY WILL HELP US LEAD TO RECOMMENDATIONS FOR CONTINUED IMPLEMENTATION OF CIRM'S STRATEGIC PLAN.

SO BASICALLY WHAT WE ARE DOING IS PROVIDING MORE GRANULARITY TO THE STRATEGIC PLAN AND HOW WE ARE GOING TO IMPLEMENT IT, AND WE ARE PROVIDING RECOMMENDATIONS TO THE BOARD TO MOVE FORWARD.

SO NEXT SLIDE. SO IN TERMS OF BACKGROUND, THE KEY HERE IS THAT CIRM NEEDS TO STRATEGICALLY ALLOCATE THE REMAINING RESOURCES TO MAXIMIZE ITS

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IMPACT BY CONSIDERING AVAILABLE FUNDS AND REVIEWING OUR PAST ALLOCATIONS. AND WHAT'S THE BACKGROUND OF ALL THIS? THE FRAMEWORK DEVELOPED OVER THE PAST MONTH IS POISED TO GUIDE US IN MAKING INFORMED DECISIONS REGARDING THE DISTRIBUTION OF OUR FUNDING. AND AS A PIONEER ENTITY IN THE REALM OF STEM CELL AND REGENERATIVE MEDICINE, CIRM'S LEGACY IS FOUNDED IN THE INVESTMENT OF SCIENTIFIC DISCOVERY TOWARDS TANGIBLE MEDICAL BREAKTHROUGHS AND ULTIMATELY CURES.

AND OUR INSTITUTE HAS BEEN INSTRUMENTAL IN FUNDING CUTTING EDGE RESEARCH AND DEVELOPING ROBUST INFRASTRUCTURE, PIONEERING EDUCATIONAL PROGRAMS, AND CATALYZING THE PROGRESSION FROM REGENERATIVE MEDICINE RESEARCH TO PRACTICAL APPLICATIONS.

AS WE WILL SEE IN THE NEXT SLIDES, THE FIELD OF REGENERATIVE MEDICINE HAS GROWN EXPONENTIALLY IN THE LAST 17 YEARS, AND CIRM HAS FINITE RESOURCES. AND GOING BACK TO LAST YEAR WHEN THIS ALL STARTED BACK IN SEPTEMBER 2023, THE SCIENCE SUBCOMMITTEE CO-CHAIR, MR. MARK FISCHER-COLBRIE, KICKED OFF A PRIORITIZATION DISCUSSION IN WHICH THE NEED FOR A STRATEGIC ALLOCATION PLAN WAS INTRODUCED.

DURING THAT MEETING MARK FISCHER-COLBRIE ASKED THE CIRM STAFF TO DEVELOP AN APPROACH AND RECOMMENDATIONS FOR PRIORITIZATION. AND THIS IS

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WHAT WE DID OVER THE LAST MONTHS. WE DEVELOPED THIS STRATEGY, AND WE PRESENTED IT TO THE BOARD BACK IN MARCH, AND THE BOARD DECIDED TO ESTABLISH THIS AS A COURSE OF ACTION MOVING FORWARD AND WITH A DEADLINE OF COMING TO THE SEPTEMBER OF 2024 MEETING WITH OUR RECOMMENDATIONS FOR PRIORITIZATION.

SO IN ORDER TO DO THAT, WE DEVELOPED THE FOLLOWING PROCESS. THIS IS THE STRATEGIC ALLOCATION FRAMEWORK THAT WE ARE PRESENTING. AND WHAT IT IS IS -- AS I MENTIONED, THESE ARE ALL IN THE MATERIALS FROM OUR MARCH ICOC MEETING AND IS A DOCUMENT THAT IS STRUCTURED AS FOLLOWS. IT HAS BACKGROUND AND RATIONALE. WHAT ARE THE REMAINING FUNDS AS OF THIS YEAR? THE REGENERATIVE MEDICINE LANDSCAPE. HOW HAVE WE DEFINED CIRM'S IMPACT TO DATE? AND THAT'S KIND OF THE BASIS OF MOVING FORWARD TO THE RECOMMENDATIONS. AND WHAT DOES THE STRATEGIC ALLOCATION FRAMEWORK CONSIST IN? AND THEN A PROPOSED TIMELINE. WITH ALL OF THIS, THE OUTPUT WILL BE THE RECOMMENDATION FOR STRATEGIC PRIORITIES.

NEXT SLIDE. THIS SLIDE PROVIDES A SNAPSHOT OF CIRM'S REMAINING FUNDS FOR THIS STRATEGIC ALLOCATION. THE TOTAL RESEARCH BUDGET AUTHORITY FROM PROP 71 AND 14 IS $7.64 BILLION. THIS IS ACTUALLY THE NET OF OPERATIONAL AND

COMPLIANCE OVERSIGHT COSTS FROM THE 8.5 BILLION INITIALLY ALLOCATED BY BOTH PROPOSITIONS. AND IN TERMS OF THE CURRENT FUNDS ALLOCATION, CIRM HAS A REMAINING BALANCE OF $3.54 BILLION. THIS IS ACCOUNTING ALL WHAT WE HAVE EXPENDED, SCHEDULED PAYMENTS AND APPROVED ALLOCATIONS, TO DATE.

WITHIN THIS REMAINING BALANCE, THERE ARE TWO SPECIFIC EARMARKS. ONE IS FOR THE NEURO RESEARCH. WE HAVE $1.11 BILLION IN FUNDS SPECIFICALLY DEDICATED AT LEAST FOR NEURO AND 93.56 MILLION THAT HAVE BEEN ALLOCATED TO INITIATIVES THAT PERTAIN TO THIS GROUP AND AIM TO IMPROVE ACCESSIBILITY AND AFFORDABILITY OF TREATMENTS DEVELOPED FROM FRUITS OF OUR FUNDING AND RESEARCH AND DEVELOPMENT.

THIS FINANCIAL REPORT BASICALLY SETS THE STAGE FOR CIRM TO DELIBERATE ON STRATEGIC ALLOCATION DECISIONS THAT WILL SHAPE OUR FUTURE, BALANCING THE DRIVE FOR INNOVATION WITH IMPERATIVE FOR TREATMENTS TO BE BOTH ACCESSIBLE AND, IMPORTANTLY, AFFORDABLE AS WELL. NEXT SLIDE.

THIS IS A QUICK OVERVIEW OF THE LANDSCAPE OF HOW THE LANDSCAPE OF REGENERATIVE MEDICINE HAS BEEN EXPERIENCING A PROFOUND AND RAPID EXPANSION. THE DATA SHOWN IS FROM THE ALLIANCE FOR REGENERATIVE

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MEDICINE ANNUAL REPORT, DATA REPORT, OF 2022, AND THE AMERICAN SOCIETY OF GENE AND CELL THERAPY, ASGCT, WHICH THEY JUST MET RIGHT NOW PAST WEEK, QUARTERLY REPORT OF Q2 2021.

AND THIS SLIDE UNDERSCORES THE EXPONENTIAL GROWTH WITNESSED IN THE SECTOR SINCE 2005, MARKING A TRAJECTORY OF ACCELERATED ADVANCEMENTS IN STEM CELL AND GENETIC THERAPIES. THIS EXPANSION IS EVIDENCED ACROSS VARIOUS PARAMETERS. IT'S VERY SMALL IN HERE, BUT I CAN TELL YOU THAT IT SHOWS THE SURGE IN THE NUMBER OF COMPANIES ENGAGED IN THESE THERAPIES, THE PORTFOLIO OF NEW PRODUCTS, AND THE GROWING NUMBER OF ACADEMIC FACULTY DEDICATED TO THE RESEARCH, AS WELL AS A ROBUST PIPELINE OF R&D PROJECTS AND CLINICAL TRIALS.

THE FIRST CHART ON THE LEFT ILLUSTRATES THERE'S BEEN A STEEP INCREASE IN PUBLICATIONS RELATED TO STEM CELLS, GENE THERAPY, OR CELL THERAPY, WHICH IS A TESTAMENT TO THE GROWING INTEREST AND INVESTMENT IN THESE FIELDS.

THE MIDDLE AND RIGHT GRAPHS DELINEATE THE EXPANDING PIPELINES FOR GENE THERAPIES AND NONGENETICALLY MODIFIED CELL THERAPIES CORRESPONDINGLY. AND EACH BAR ON THE MIDDLE AND RIGHT REPRESENT A SNAPSHOT OF ACTIVE PROGRAMS AND

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REFLECT NOT ONLY THE INITIATION OF PRECLINICAL AND PHASE 1 TRIALS, BUT ALSO THE PROGRESSION TO MORE ADVANCED STAGES OF CLINICAL TESTING.

THIS LANDSCAPE BRINGS TO THE FRONT FOR US A VERY COMPELLING NARRATIVE, THAT THE FIELD OF REGENERATIVE MEDICINE IS NOT JUST GROWING, BUT IS STRIDING AT A PACE THAT REQUIRES A STRATEGIC AND THOUGHTFUL ALLOCATION OF FUNDS.

AND WITH THIS KIND OF LANDSCAPE, THE IMPLICATIONS FOR HEALTHCARE ARE IMMENSE, AND IT UNDERSCORES THE IMPORTANCE THAT WE HAVE AS AN ORGANIZATION TO PROVIDE THE STRATEGIC FUNDING TO LEAD TO THE MOST PROMISING AVENUES OF RESEARCH THAT CAN TRANSLATE INTO LIFE-ALTERING TREATMENTS FOR PATIENTS.

THE NEXT SLIDE PROVIDES A VERY QUICK OVERVIEW OF CIRM'S IMPACT TO DATE. AND IT HAS BEEN REALIZED THROUGH THESE FOUR LARGE INITIATIVES THAT INTEROPERATE TOGETHER TO REALIZE OUR MISSION. THE KEY AREAS OF EMPHASIS ARE, THE FIRST ONE, DEVELOPMENT OF CELL AND GENE THERAPIES. THIS IS ALIGNED WITH ONE OF THE IMPACT GOALS AS WELL.

SECOND ONE IS COLLABORATIVE NETWORKS FOR DISCOVERY RESEARCH. WE HAVE BEEN FOSTERING THIS COLLABORATIVE ECOSYSTEM, AND THERE IS AN OPPORTUNITY

TO REALLY BUILD ON THAT. THAT COULD ALSO BE ALIGNED WITH ANOTHER IMPACT GOAL.

THE THIRD ONE IS TRAINING AND WORKFORCE DEVELOPMENT, AND WE'VE BEEN VERY SUCCESSFUL WITH OUR EDUCATIONAL INITIATIVES AND DEVELOPMENT OF WORKFORCE. AND WE ARE THINKING ABOUT WAYS THAT WE CAN ENHANCE THIS.

AND THE LAST ONE IS ADVANCEMENTS IN REGENERATIVE MEDICINE TECHNOLOGIES. WE HAVE NOT SO FAR BEEN FOCUSING ON ACCESSIBILITY AND AFFORDABILITY. THIS IS PROP 14 SO FAR MEANING LIKE UP UNTIL NOW, UP UNTIL PROP 14. PROP 14 HAS A VERY CLEAR MANDATE FOR THIS, AND IT'S ACTUALLY WHAT'S GOING TO BE THE FOCUS OF TODAY'S MEETING.

SO ALL THESE IMPACT -- THE RECOMMENDATIONS THAT WE ARE GOING TO MAKE LEAD TOWARDS DETERMINING THE IMPACT OF CIRM MOVING FORWARD. NEXT SLIDE.

AS WE PRESENTED DURING THE MARCH ICOC AND AS WE MOVE ON TO THE NEXT CRITICAL COMPONENT OF OUR STRATEGIC PLANNING, WE FACE THE PIVOTAL DESIGN QUESTIONS AT THE HEART OF OUR STRATEGIC ALLOCATION FRAMEWORK. THE FIRST ONE IS HOW CAN CIRM MAKE THE GREATEST IMPACT ON ITS MISSION? THE SECOND ONE IS HOW MIGHT CIRM EFFECTIVELY ALLOCATE ITS REMAINING BUDGET OF $3.54 BILLION. AND THERE IS A SUBQUESTION

WHICH HAS TO DO WITH THE NEURO BUDGET THAT'S BEEN BUILT THROUGH THE SCIENCE SUBCOMMITTEE AND THE NEURO TASK FORCE. AND IT'S HOW WE WILL ALLOCATE THIS 1.11 BILLION IN NEURO.

SO BASED ON THOSE HIGH LEVEL DESIGN QUESTIONS, WE MOVED FORWARD TOWARDS THE STRATEGIC ALLOCATION FRAMEWORK. NEXT SLIDE. SO THIS SLIDE REPRESENTS THE PROCESS THAT THE CIRM STAFF IN COLLABORATION WITH DIFFERENT BODIES OF THE BOARD AND WORKING GROUPS SUCH AS YOURS HAS BEEN UNDERGOING. THIS IS, AS I MENTIONED EARLIER ON, A STRUCTURED AND DATA DRIVEN PROCESS TO PRIORITIZE RESOURCE ALLOCATION AND PROVIDE FURTHER GRANULARITY IN TERMS OF IMPACT GOALS AND THEIR SUCCESS MEASURES WHICH ULTIMATELY WILL LEAD TO THESE RECOMMENDATIONS TO IMPLEMENT OUR STRATEGIC PLAN.

AND WE HAVE DIVIDED IT IN FOUR SECTIONS. THE IMPACT GOALS, ALSO STRATEGIC ALLOCATION FRAMEWORK CATEGORIES, ARE THE BEACON THAT GUIDELINES ALL OUR EFFORTS. THESE GOALS ARTICULATE THE DESIRED OUTCOMES AND MILESTONES THAT WE AIM TO ACHIEVE, ENSURING THAT EVERY DOLLAR ALLOCATED MOVES US CLOSER TO OUR VISION. AND DURING TODAY'S PRESENTATION, THE GOAL WILL BE PRESENTED AS CATEGORIES WHICH ARE BASICALLY A PROXY TO THE IMPACT GOALS. AND THE

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REASON FOR THIS I'LL MENTION LATER IS BECAUSE THE GOALS HAVE AN ITERATIVE PROCESS. AND UP UNTIL WHEN WE PRESENT THEM IN JUNE, WE ARE PRESENTING THEM AS CATEGORIES.

NEXT WE HAVE THE GUIDING QUESTIONS. IN ORDER TO KNOW WHAT RECOMMENDATIONS WE NEED TO MAKE TO LEAD TO AN IMPACT GOAL WITH MILESTONES AND OUTCOME MEASURES, WE NEED TO DEFINE QUESTIONS. AND THE MEMO THAT WE SHARED HAD THE QUESTIONS FOR THE GOAL NO. 2, WHICH IS THE GOAL THAT PERTAINS TO THIS WORKING GROUP. NOW, THOSE GUIDING QUESTIONS, IN ORDER TO ANSWER THEM, WE NEED TO COLLECT DATA AND ANALYZE IT. SO WE HAVE ALSO PROPOSED WHAT DATA WE NEED TO COLLECT IN ORDER TO ANALYZE TO MAKE THOSE RECOMMENDATIONS.

SO WHAT WE WILL BE GOING THROUGH IN THE NEXT SLIDES ARE WHAT PERTAINS TO TODAY'S MEETING. NEXT SLIDE. SO THESE ARE THE FOUR CATEGORIES VERY HIGH LEVEL. WE HAVE THE CELL AND GENE THERAPY APPROVALS. THIS WOULD HAVE TO DO WITH PROPELLING CELL AND GENE THERAPY FOR PREVALENT AND RARE DISEASES TO A SUCCESSFUL OUTCOME. AND THE GOALS THERE ARE DEFINED WITH MEASURABLE, TANGIBLE OUTCOMES THAT WE WILL NOT DISCUSS TODAY.

THE SECOND IS THE ACCESSIBILITY AND

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AFFORDABILITY OF CIRM-FUNDED CELL AND GENE THERAPIES WHICH IS WHAT WE ARE GOING TO DISCUSS TODAY. AND THEN WE HAVE ANOTHER GOAL THAT HAS TO DO WITH DISCOVERY OF NOVEL MECHANISMS. THIS HAS TO DO WITH THE DISCOVERY ECOSYSTEM AND COLLABORATIVE ECOSYSTEM AND DATA INFRASTRUCTURE. AND THEN THE DIVERSE WORKFORCE DEVELOPMENT.

NEXT SLIDE IS JUST TO HIGHLIGHT THE ACCESSIBILITY AND AFFORDABILITY CATEGORY. AND NOW ON SLIDE 14 WHAT WE HAVE IS THE MAIN POINT OF DISCUSSION FOR TODAY, THE MAIN SLIDE THAT FRAMES THE DISCUSSIONS FOR TODAY.

SO FOR TODAY'S DISCUSSION, WE ARE ACTUALLY ALREADY BRINGING THE GOAL IS GOAL NO. 2, WHICH IS THE GOAL THAT THIS WORKING GROUP SHOULD BE EVALUATING, IS A GOAL OF ENSURING THAT EVERY CIRM-FUNDED PROJECT COMPLETING ADVANCED CLINICAL TRIALS HAVE A STRATEGY THAT ENABLES ACCESSIBILITY AND AFFORDABILITY BY ALL CALIFORNIA PATIENTS, PARTICULARLY UNDERSERVED POPULATIONS, THE GOAL THAT WE WANT TO GET TO. IS THIS GOAL (UNINTELLIGIBLE)? IS THIS FEASIBLE? AND THAT'S WHAT WE WILL HAVE FOR DISCUSSION.

AND DURING -- IN THE MEMO WE PROVIDED SOME MORE GRANULAR QUESTIONS. WHAT IS THE LANDSCAPE OF

ACCESSIBILITY AND AFFORDABILITY FOR CELL AND GENE THERAPIES? SO THESE QUESTIONS COULD BE THE ONES THAT BY ANSWERING WE COULD BE ABLE TO DEVELOP RECOMMENDATIONS THAT WILL HELP US LEAD TO THAT GOAL. AND THAT'S HOW WE CAN PRESENT IT IN SEPTEMBER TO THE BOARD.

SO WHAT WE NEED TO HAVE IN TODAY'S MEETING IS AN EVALUATION OF WHETHER THE HIGH LEVEL QUESTIONS THAT WE HAVE POSED HERE AND THE GOAL AS IT IS DEFINED REPRESENT WHAT THIS WORKING GROUP THINKS IS THE MOST APPROPRIATE GOAL FOR CIRM AND FOR THIS GROUP TO DEAL WITH.

NOW, I'M NOT GOING TO GO OVER IT BECAUSE THAT'S GOING TO BE THE MAIN DISCUSSION SLIDE. WHAT I'M GOING TO SHOW NOW IS VERY HIGH LEVEL THREE SLIDES THAT SHOW THE TIMELINE AND HOW ARE WE GOING TO COORDINATE THIS WITH EVERYTHING ELSE. AND THEN WE WILL COME BACK TO SLIDE 14, AND I WILL LET MY COLLEAGUE GEOFF LOMAX AND CO-CHAIR BONNEVILLE TO LEAD THAT DISCUSSION.

OKAY. SO SLIDE NO. 15, THIS SHOWS WHERE WE ARE TODAY, MAY 14 OF 2024. AND WE HAVE, WE HAVE GEARING TOWARDS SEVERAL IMPORTANT DEADLINES FOR PRESENTATION OF OTHER, THE SAME THING, BUT RELATING TO OTHER GOALS. SO AT THE JUNE 14, IT'S NOT JUNE

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4TH, IT'S JUNE 14, 2024, ABOUT A WEEK BEFORE THE ICOC, WE WILL BE PRESENTING TO THE JOINT NEURO TASK FORCE AND SCIENCE SUBCOMMITTEE THE GOAL PERTAINING TO -- WE WILL BE DISCUSSING THE NEUROSCIENCE FOCUS, BUT I'M NOT GOING TO GO INTO ALL OF THIS. THIS IS JUST TO SHOW THAT WE HAVE A LOT OF STAGGERED ACTIVITIES. AND THE NEXT TIME THAT WE WILL BE MEETING, GATHERING WITH THIS GROUP, IT WILL BE ON AUGUST 7TH. AT THAT TIME WE ARE GOING -- AND YOU CAN GO TO THE NEXT SLIDE. SO THE AUGUST 7TH COULD BE IN PREPARATION FOR THE FINAL DISCUSSION WITH THE SCIENCE SUBCOMMITTEE ON THE SEPTEMBER 13TH OF ALL THE GOALS. AND THEN ON SEPTEMBER 26TH IS THE FINAL MEETING WHERE WE WILL BE PRESENTING THE FINAL RECOMMENDATIONS WITH THE GOALS TO THE BOARD.

NOW, BETWEEN SEPTEMBER AND DECEMBER IS WHAT WE HAVE GIVEN OURSELVES OF TIME TO PROVIDE THE GRANULAR, ONCE WE GET THE FEEDBACK FROM THE BOARD AND THE APPROVAL TO MOVE FORWARD, THEN WE WILL BE REVISING CONCEPTS AS APPROPRIATE AND IF IT PERTAINS AND THE STRUCTURE OF HOW THE PROGRAMS WILL BE MOVING FORWARD. SO THAT'S THE WHOLE PROCESS.

THE NEXT SLIDE IS ANOTHER HIGH LEVEL SLIDE THAT SHOWS WHAT ARE WE EXPECTING AT EVERY MEETING. SO TODAY'S MEETING IS NOT REFLECTED HERE, BUT THE

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AUGUST ACCESSIBILITY AND AFFORDABILITY WORKING GROUP, AS YOU CAN SEE ON THE FIFTH ROW, SHOWS TO PRESENT UPDATES ON GOAL 2 AND DISCUSS ASSOCIATED DATA AND DISCUSS POTENTIAL RECOMMENDATIONS FOR GOAL 2. SO BETWEEN TODAY, WHEN WE WILL DISCUSS THE QUESTIONS AND THE GOAL AS WE HAVE PRESENTED, AND AUGUST WE WILL HAVE TO HAVE GATHERED THE DATA, ANALYZED IT, AND THEN COME IN AUGUST WITH THIS GROUP TO DISCUSS THE DATA AND THE POTENTIAL RECOMMENDATIONS TO MAKE SURE THAT WE ARE ALL ALIGNED BEFORE WE GO TO THE SEPTEMBER SCIENCE SUBCOMMITTEE BEFORE THE ICOC. HOPEFULLY THAT WASN'T TOO MUCH INFORMATION. IT'S HOPEFULLY CLEAR, BUT WE ARE HAPPY TO ANSWER ANY QUESTIONS.

SO I COULD COME NOW BACK TO THE SLIDE NO. 14, AND I WOULD LIKE TO LEAVE TO MY COLLEAGUE DR. LOMAX AND CO-CHAIR BONNEVILLE TO LEAD THIS DISCUSSION.

CHAIRPERSON BONNEVILLE: THANK YOU, ROSA, SO MUCH. GEOFF, DO YOU WANT TO START WITH THE QUESTIONS, AND THEN WE'LL LEAD INTO SOME COMMENTS THAT HAVE BEEN SUBMITTED BY OTHERS?

DR. LOMAX: SURE. SO YOU HAVE THE QUESTIONS IN THE SLIDE. WE HAVE A BROADER SET OF QUESTIONS ON GOAL 2 IN THE BRIEFING MEMO THAT'S ON

PAGE 2 AND 3. AND I THINK WE'VE CIRCULATED THAT. SOME OF THE MEMBERS THAT AREN'T AVAILABLE FOR TODAY'S MEETING HAVE PROVIDED SOME INITIAL COMMENTS. AND I THINK PERHAPS WE CAN USE THOSE AS AN ICEBREAKER TO LEAD INTO THE DISCUSSIONS.

CHAIRPERSON BONNEVILLE: GREAT. SO MANY OF YOU, I'M SURE, SAW HARLAN'S EMAIL. AND HE RESPONDED TO THE MEMO AND TO THE PRESENTATION. HE COULDN'T BE HERE TODAY. HE'S TRAVELING. SO I'M JUST GOING TO SHARE HIS THOUGHTS AND READ THEM INTO THE RECORD.

"I THINK IT'S NOT REALISTIC FOR RESEARCHERS CONDUCTING LATER STAGE RESEARCH BASED ON CIRM FUNDING TO FULLY UNDERSTAND THE CLINICAL IMPACT OF A PRODUCT BEFORE THE STUDIES ARE COMPLETE AND FINALIZED. AS SUCH, IT MAY BE DIFFICULT TO DESCRIBE THE OPTIMAL WAY TO ACCESS IN THE CLINICAL TRIAL DESIGN. IT MAY COME ACROSS AS AN ADDED BURDEN DURING A PHASE WHERE THE FOCUS IS DEFINING EFFICACY.

"SO PERHAPS RESEARCHERS CAN ADDRESS AND OUTLINE THE ISSUES OF UNMET NEEDS AND SPECIFICALLY CALL OUT UNDERREPRESENTED GROUPS AND HOW DISCOVERY OR DRIVE MAY FAVORABLY IMPACT SUCH POPULATIONS OR THE PLAN FOR LOCATING CLINICAL TRIAL SITES OR OUTREACH TO CLINICS SHOULD TAKE INTO ACCOUNT THE

NEED TO BE LOCATED IN UNDERREPRESENTED COMMUNITIES.

"SECOND, I SUGGEST WE FOCUS ON REGULATORY AND LEGISLATIVE CHANGES THAT WOULD ENSURE THAT ALL POPULATIONS HAVE APPROPRIATE ACCESS TO A NEW DRUG BASED ON WHERE IT SITS IN TREATMENT ALGORITHMS ESTABLISHED BY EXPERTS IN THIS FIELD. MANDATE THAT EVERY HEALTHPLAN OR RISK BEARING NETWORK HAS AT LEAST ONE ACADEMIC CENTER IN ITS NETWORK THAT CAN ADMINISTER THE DRUG IF AND WHEN FDA APPROVED AND IS AFFILIATED WITH AT LEAST ONE MAJOR RESEARCH CENTER WORKING WITH CIRM SO ITS MEMBERS CAN ACCESS ALL STAGES OF CLINICAL TRIALS.

"REQUIRE ALL MANAGED CARE ENTITIES AND RELATED NETWORKS TO EDUCATE THESE PHYSICIANS ON NEW ENTRANTS OF APPROVED DRUGS AND TRIALS AVAILABLE THROUGH CIRM RESEARCH CENTERS AND NETWORKS AND THAT THEY REPORT ON THIS PUBLICLY.

"ALSO, PERHAPS THERE COULD BE SOME OBLIGATION ON THE IP LICENSING ENTITY TO HAVE A PLAN FOR ACCESS. THIS USUALLY REQUIRES COOPERATION WITH HEALTHPLANS. WOULD REQUIRING PHARMA TO HAVE A PATIENT ASSISTANCE PROGRAM IN PLACE FOR COPAY, COINSURANCE HELP? ADDITIONALLY, THERE COULD BE A REQUIREMENT THAT COMMERCIALIZING ENTITIES OFFER AN OUTREACH PROGRAM TO EDUCATE DOCTORS.

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"WE NEED TO ADDRESS THE FACT THAT PAYORS WILL WANT TO LIMIT REIMBURSEMENT TO PROVIDERS OR PHARMA. WE NEED TO BE SURE PROVIDERS DO NOT HAVE THE ECONOMIC DISINCENTIVES FOR APPROPRIATE USE OF NEW DRUGS. AND WE SHOULD DEVELOP DISTRIBUTION CHANNELS THAT LIMIT OR ELIMINATE THIRD-PARTY MIDDLEWARE THAT DOES NOT ADDRESS PATIENT VALUE.

"MY GENERAL THOUGHTS ARE THAT WE WANT TO AVOID ADDING TOO MUCH COMPLEXITY FOR THE RESEARCH. WE SHOULD NOT BURDEN RESEARCHERS TO SOLVE COMPLEX, MULTIDIMENSIONAL, MULTIFACTOR ISSUES, AND WE NEED THE PAYORS AND PROVIDERS TO EMBRACE THE EMERGING TECHNOLOGY AND ASK PHARMA TO BETTER EDUCATE IN UNDERSERVED AREAS AND PERHAPS OFFER REDUCING UNDERSERVED AREAS IF THE BENEFIT FLOWS THROUGH TO PATIENTS AND ALLEVIATES PROVIDER BURDEN."

SO I WANT TO OPEN THIS UP TO MEMBER CONVERSATION. SO IF YOU WOULD RAISE YOUR HANDS, WE CAN CALL ON YOU ACCORDINGLY.

GEOFF, DO YOU WANT TO COMMENT ON A COUPLE OF HARLAN'S POINTS THAT WE DISCUSSED EARLIER?

DR. LOMAX: YEAH. WOULD APPRECIATE IT. THANKS.

AND SO I THINK THE FIRST POINT ABOUT SORT OF THE FEASIBILITY OF RESEARCHERS WHO ARE CONDUCTING

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LATE STAGE TRIALS, SORT OF THAT IMPACT PIECE, I THINK THERE ARE SOME DEVELOPMENTS THAT ARE HELPFUL. I THINK THERE'S AN UNDERSTANDING OF THAT POINT IN THE SORT OF CLINICAL DEVELOPMENT PATHWAY. AND ONE OF THE MORE RECENT DEVELOPMENTS ON THE CIRM SIDE IS THE ABILITY OF A LATE STAGE CLINICAL PROGRAM TO ACCESS WHAT WE'VE NOW CHARACTERIZED AS THE CLIN4 FUNDING, WHICH WE PROVIDED A BACKGROUND ON THAT PROGRAM IN EMAIL CORRESPONDENCE.

THE CLIN4 PROGRAM SPECIFICALLY ALLOCATES FUNDING TO ADDRESS ISSUES THAT WOULD BE NECESSARY TO, FOR EXAMPLE, IF THEY WERE SEEKING TO GET REIMBURSEMENT, IF THEY WERE EMBARKING ON A PROGRAM OF SORT OF EVIDENCE DEVELOPMENT, WHICH IS TYPICALLY REQUIRED BY CMS. IT'S QUITE FREQUENT THAT THEY WILL GIVE AN APPROVAL FOR A PRODUCT, BUT THEY WILL REQUIRE ONGOING EVIDENCE DEVELOPMENT. AND THAT THAT EVIDENCE COULD BE USED TO MAKE DETERMINATIONS FOR FACTORS SUCH AS DURABILITY, EFFICACY, SAFETY, AND LONG-TERM PATIENT OUTCOME STUDY. SO IT'S THOSE SORTS OF ACTIVITIES WHICH I THINK, TO DR. LEVINE'S POINT, THEY WOULDN'T NECESSARILY BE PREPARED TO DO IN SORT OF THE EARLY STAGE CLINICAL DEVELOPMENT. BUT AS THEY MOVE THROUGH THAT DEVELOPMENT PATHWAY, THEY COULD ON THE BACK END DEVELOP THAT EVIDENCE

THERE.

AND I THINK THAT THE OTHER POINT, I THINK, WAS USEFUL, ONE OF THE QUESTIONS WE HAVE FOR GOAL 2 ARE A SET OF QUESTIONS AROUND POLICY DEVELOPMENT AND GENERAL POLICIES AND RESOURCES THAT CIRM COULD HELP FACILITATE. AND AS A REMINDER, ROSA POINTED OUT THIS WORKING GROUP HAS RESOURCES TO DEPLOY SHOULD WE NEED TO DO POLICY RESEARCH OR POLICY DEVELOPMENT. AND HE SORT OF ITEMIZED SOME SPECIFIC TYPES OF POLICY CHANGES. AND I KNOW THEY'VE BEEN VERY ACTIVE IN ADVOCACY IN CALIFORNIA. SO I THINK THAT THE QUESTION FOR THE WORKING GROUP IS TO WHAT EXTENT SHOULD CIRM REALLY BE FOLLOWING EXISTING POLICY DEVELOPMENT IN CALIFORNIA ADVOCACY AND SORT OF PURSUING THOSE IN LINE WITH SORT OF THE STRATEGIC ALLOCATION FRAMEWORK OR SORT OF BROADER GENERAL ISSUES NECESSARY FOR PATIENT ACCESS FOR THESE TYPES OF TREATMENTS.

SO I THINK HIS COMMENTS ARE VERY USEFUL IN THE SENSE THEY START TO FIT INTO SOME OF THESE CATEGORIES, QUESTION CATEGORIES, WE'VE OUTLINED IN THE BRIEFING MEMO.

SO WITH THAT SAID, I AGAIN WOULD SORT OF INVITE WORKING GROUP MEMBERS IF THEY HAVE COMMENTS OR THOUGHTS WHERE THEY WANT TO EXPAND ON.

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CHAIRPERSON BONNEVILLE: THANK YOU. AND, ROSA, YOU WILL COME BACK TO THIS WORKING GROUP IN AUGUST WITH SOME OF THE DATA THAT YOU'VE GATHERED SPECIFICALLY AROUND THE QUESTIONS BOTH FOR IN GENERAL, BUT SPECIFIC TO THIS WORKING GROUP AS WELL?

DR. CANET-AVILES: CORRECT. IF THERE IS NO MORE FEEDBACK, WHAT WE ARE GOING TO DO IS WE ARE GOING TO TAKE THE QUESTIONS THAT WE DEVELOPED, AND WITH GEOFF AND COLLEAGUES, WE ARE GOING TO DETERMINE THE DATA THAT IS LOGICALLY NECESSARY TO ANSWER THOSE QUESTIONS. HE WILL PROBABLY BE IN CONTACT WITH YOU AND THE WORKING GROUP TO MAKE SURE THAT THE DATA, THEY ARE ALL IN AGREEMENT, AND THE GOAL WOULD BE THAT IN AUGUST, BECAUSE WE WILL BE LIKE A MONTH BEFORE SEPTEMBER, WE WILL BE COMING WITH THE RECOMMENDATION AND THE ANALYSIS AND PRESSURE TEST BEFORE GOING TO THE SCIENCE SUBCOMMITTEE AND THE BOARD.

CHAIRPERSON BONNEVILLE: GREAT. THANK YOU. ADRIANA HAS A QUESTION. SO I'M GOING TO CALL ON ADRIANA.

DR. PADILLA: THANK YOU. I'M LOOKING AT HARLAN'S SUMMARY, AND I AGREE IT'S VERY COMPLEX. AND RESEARCHERS DON'T REALLY UNDERSTAND A LOT OF THE -- I MEAN THEIR FOCUS IS ON JUST TRYING TO GET

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TO THE EFFICACY AND PROCESS OF THEIR RESEARCH. BUT WE ALREADY ARE ASKING THAT. MY QUESTION IS WHAT IS -- WHAT ARE THE DIFFICULTIES THAT WE SEE NOW IN THE CURRENT PROCESS THAT WE HAVE BECAUSE THEY ARE BEING ASKED WHAT ARE THE CLINICAL IMPLICATIONS.

CHAIRPERSON BONNEVILLE: YES.

DR. PADILLA: AND HOW DO THEY ANTICIPATE WORKING WITH POPULATIONS THAT MAY NOT BE ABLE TO ACCESS THEIR STUDIES AND THEIR OUTCOME GOAL? AND SO WE HAVE ALL THIS RESEARCH THAT HAS BEEN APPROVED, FUNDED FOR THESE PARTICULAR ISSUES. WHAT HAVE BEEN THE CHALLENGES THAT THE TEAM HAS SEEN SO FAR THAT MAKES IT PROBLEMATIC FOR OUR FOCUS FOR FUNDING MUCH MORE STRATEGIC?

CHAIRPERSON BONNEVILLE: THANK YOU, ADRIANA. I THINK ABLA CAN SPEAK TO THAT.

DR. CREASEY: THANK YOU, ADRIANA, FOR THE QUESTION. FOR THE LAST COUPLE OF YEARS, WE'VE BEEN REQUIRING, AS YOU KNOW, A DIVERSITY, EQUITY, AND INCLUSION PLAN FOR EVERY TRIAL. AND INVARIABLY PEOPLE HAVE BEEN COMPLIANT IN GIVING US, ESPECIALLY DEPENDING ON THE THERAPY AND THE DISEASE. WE HAVE HAD GOOD CHANGES IN THE SENSE THAT IF THE OPPORTUNITY ALLOWS INCLUSION MORE -- THEY'RE REACHING OUT TO GROUPS THAT ARE UNDERSERVED. THE

DATA ARE STILL SMALL. WE DON'T HAVE ENOUGH DATA TO LIKE SHOW IN GRAPHS ET CETERA, BUT WE ARE ENCOURAGED.

ONE OF THE TOPICS I JUST NEED TO REMIND YOU OF IS MANY OF OUR TRIALS ARE ALSO IN RARE DISEASES. AND SOME OF THOSE RARE DISEASES AFFECT SOME OF THE DIVERSITY, EQUITY, INCLUSION TYPE PATIENTS, THE FOLKS WHO ARE UNDERSERVED, BUT WE ONLY HAVE AN N OF LIKE, SMALL N'S PER TRIAL. AND SO I THINK WITHIN THE NEXT YEAR WE SHOULD HAVE ENOUGH INFORMATION TO ALLOW US TO REALLY FIGURE OUT HOW TO PIVOT. SHOULD WE DO CHANGES ALSO IN OUR APPLICATIONS? I PERSONALLY AM ENCOURAGED BY HOW PEOPLE ARE RESPONDING TO THAT PARTICULAR PART OF THE GRANT, AND THEY'RE SHOWING THAT MANY -- WHENEVER POSSIBLE, DEPENDING ON THE DISEASE, THEY ARE REACHING OUT TO FOLKS WHO ARE UNDERSERVED AND INCLUDING THEM IN THE TRIAL RECRUITMENT AND ENROLLMENT.

DR. LOMAX: IF I CAN ADD TO ABLA SOME MORE ON THE POLICY SIDE. WE'RE KIND OF AT A UNIQUE MOMENT. FIRST OF ALL, WE CIRCULATED THE WHITE PAPER THAT I THINK IS EXTREMELY USEFUL IN TERMS OF GETTING A SORT OF JUST-IN-TIME VIEW OF THE REIMBURSEMENT LANDSCAPE. THAT WAS THE WHITE PAPER BY NEW DIGS

AND --

CHAIRPERSON BONNEVILLE: ICER.

DR. LOMAX: ICER. THANK YOU FOR THAT. AND I THINK IN THERE WHAT REALLY COMES OUT IN THAT DOCUMENT IS WE'RE JUST, AND I THINK THIS IS PARTICULARLY IN THE CONTEXT OF SOME OF THE GENE THERAPIES, JUST SEEING THAT EMERGENT REIMBURSEMENT LANDSCAPE, PARTICULARLY ON THE PUBLIC PAYOR SIDE, AND AS THAT LANDSCAPE IS BEGINNING TO EMERGE, THERE IS A PILOT PROGRAM THAT CMS HAS LAUNCHED. WHAT THAT PROGRAM IS SORT OF TACKING TOWARDS, RATHER THAN PARTICULARLY ON THE PUBLIC PAYOR SIDE, LOOKING AT THESE THERAPEUTICS OR THESE TREATMENT OPPORTUNITIES IN SORT OF A ONE-OFF SORT OF WAY DEVELOPING A MORE COMPLETE FRAMEWORK FOR HOW THEY'RE GOING TO PLAN AND FINANCE THESE PROGRAMS WHICH WILL START TO GIVE US SOME SENSE OF SCALE AND POTENTIALLY OTHER DATA POINTS.

THEN FROM THE PROGRAM SIDE WITH THE THERAPEUTICS DEVELOPMENT SIDE, THE DATA GENERATED THROUGH THAT THERAPEUTIC PROGRAM PRESUMABLY CAN THEN ALIGN WITH THOSE POLICIES. SO THEY'RE MOVING AGAIN FROM SORT OF TAKING THEM AS THEY COME TO A MORE DEFINED FRAMEWORK FOR FINANCING THESE POPULATIONS. SO IN THAT SENSE I WOULD SUSPECT IT WILL BE A LOT OF

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WORK ON OUR PART TO REALLY TRACKING HOW THAT DEVELOPS. I THINK THAT WORK IS GOING TO HAPPEN IN CALIFORNIA, SO WE NEED TO BE PAYING CLOSE ATTENTION TO THAT.

DR. CREASEY: AND THAT SPEAKS TO THE FACT THAT THERE IS A NEED FOR THERAPEUTICS DEVELOPMENT AND ACCESSIBILITY AND AFFORDABILITY IMPROVED TO ALIGN TOGETHER, WORK CLOSELY, BOTH AS POINTED OUT BY HARLAN, THAT THERE NEEDS TO BE A REGULATORY AND LEGISLATIVE CHANGES. AND THAT'S WHAT WE'RE ALSO WORKING TOWARDS ESPECIALLY, AGAIN, FOR WHERE THE DISEASES, AT LEAST THE INDICATIONS, FOR EXAMPLE, IN GENE THERAPY HAVE LED TO AN APPROVED DRUG, AND DO WE HAVE ANY SIMILAR DRUGS IN OUR PIPELINE AND HOW ARE WE GOING TO COORDINATE ALL THAT TO MAKE THAT HAPPEN.

DR. PADILLA: IN GENERAL, THEN, WE'RE NOT ASKING ANYTHING MORE OF THE RESEARCHERS; IS THAT CORRECT?

DR. CREASEY: WE ARE IN THE SENSE THAT ONCE WE KNOW MORE ABOUT THE POLICY PART AND THE PAYORS ARE EXPECTING. AND SO WE'RE LEARNING MORE FROM THAT SIDE FIRST SO THAT WE CAN AMEND OUR REQUIREMENTS FOR THE APPLICATIONS. SO THAT'S WHAT WE'RE DOING.

DR. PADILLA: OKAY. SO THE QUESTION THEN

IN THE STEP PROCESS, I MEAN HARLAN'S RESPONSE WAS VERY DIRECT TO THE QUESTION, IS THAT IT STATES THAT THERE'S A REQUIREMENT FOR THE RESEARCHERS TO ACTUALLY HAVE EXPERTISE IN FINANCING AND EVERYTHING WHEN THAT'S NOT EVEN AVAILABLE AT THIS TIME. SO MAYBE THE QUESTION NEEDS TO BE REFORMATTED IN ORDER TO NOT HAVE THAT CONFUSION IN PLACE.

DR. CREASEY: ADRIANA, IF I CAN JUST REMIND EVERYONE IN THE ROOM AND ON ZOOM THAT CLIN4 FUNDING THAT WE JUST LAUNCHED INCLUDES A LOT OF WHAT THE PAYORS ARE EXPECTING, SUCH AS INITIATION OF PRECOMMERCIALIZATION ACTIVITIES. SO THEY HAVE -- A GRANTEE WILL HAVE TO WORK WITH AGENCIES LIKE ICER AND DEVELOP ESSENTIALLY A WHITE PAPER THAT SUPPORTS WHY THEY'RE DOING AND HOW THE PRODUCT IS GOING TO BE LAUNCHED. SO ESSENTIALLY WE'RE PRIMING THEM TO BE READY FOR POLICY AND FOR THE PAYOR'S READINESS. AND THAT'S THROUGH, AGAIN, THE CLIN4. WE HAVE NOT HAD YET ANY CLIN4 APPLICANTS. WE'RE OPTIMISTIC, BUT WE'RE ESSENTIALLY PREPARING THEM ALSO THROUGH AMENDMENTS IN THE CLIN2 AND THE CLIN4. AND I'LL POINT THAT OUT AGAIN IN MY PORTION OF THIS SESSION.

BUT PERSONALLY AS A PERSON WHO HAS WORKED ON BOTH SIDES, I THINK WE ARE READY TO MAKE SURE THAT WHATEVER YOU AND HARLAN ARE KIND OF APPEALING

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TO AND FOR US TO EDUCATE OUR APPLICANTS, WE'RE ALMOST THERE. WE'RE NOT THERE YET, BUT WE'RE ALMOST THERE.

AND I RECALL A CONVERSATION WITH SACRAMENTO REGARDING THEIR INTEREST WHEN IT COMES TO MEDICAID, MEDICARE. PEDIATRICS INDICATIONS WAS TOP ON THE LIST. AND WE HAVE A LOT OF PEDIATRIC DISEASE INDICATIONS. AND SO THERE ARE, LIKE I SAID, THE POSSIBILITY OF ALIGNMENT IS THERE IN THE NEAR FUTURE.

DR. PADILLA: OKAY. SO THEN IT'S MORE LIKE A PROCESS -- OUTCOME WAITING TO HAPPEN, BUT CURRENTLY THE RESEARCHERS THAT ARE SUBMITTING APPLICATIONS FOR FUNDING ARE AWARE OR THEY SHOULD BE AWARE BECAUSE THEY ARE RESPONDING TO THAT QUESTION RIGHT NOW WITHOUT ALL THE FULL DATA THAT ARE IN PLANS FOR DEVELOPMENT, BUT THAT THEY SHOULD BE ANTICIPATING THAT THAT'S ONE OF THEIR GOALS THAT THEY WILL BE WORKING TOWARDS AS THIS PROCESS MOVES ON; IS THAT CORRECT?

DR. CREASEY: YES.

DR. PADILLA: OKAY.

CHAIRPERSON BONNEVILLE: THANK YOU.

DR. PADILLA: I'M FINE.

CHAIRPERSON BONNEVILLE: MAHESWARI, YOU

HAVE YOUR HAND RAISED.

DR. SENTHIL: YES. AT THE RISK OF BEING REDUNDANT, I STILL WANTED TO EMPHASIZE POINTS THAT HAVE BEEN MADE. I'M A COMMISSION, A RESEARCHER, TRIALIST, AS WELL AS A PERSON WHO RUNS A CLINICAL TRIALS UNIT. AND ALTHOUGH DR. LEVINE'S COMMENT ABOUT WE SHOULDN'T BURDEN THE RESEARCHERS TOO MUCH ABOUT COMPLEXITY, I DON'T THINK WE CAN COMPLETELY ABDICATE THEM OF THEIR RESPONSIBILITIES TO REALLY THINK ABOUT WHY THEY ARE EVEN DOING THAT RESEARCH AND WHAT IS THE LARGER IMPACT OF THE RESEARCH. AND AS WE ALL KNOW, CELL AND GENE THERAPY IS NOT ONLY FOR RARE DISEASES. IT IS NOW COMING INTO THE MANAGEMENT OF EVEN THE MORE COMMON PROBLEMS THAT WE SEE WHICH MIGHT HAVE A WIDE DEMOGRAPHIC THAT MAY BE AFFECTED.

SO RESEARCHERS ARE EXPECTED TO UNDERSTAND THE DEMOGRAPHIC DISTRIBUTION, WHERE THE PATIENTS ARE, AND THE DATA BARRIERS THAT MAY EXIST FOR THESE PATIENTS TO ACCESS THESE CLINICAL TRIALS IF IT'S BEING FUNDED THROUGH CIRM. AND ASKING FOR THEM TO HAVE A CLEAR PLAN ARTICULATED IN THEIR GRANT IS VERY MUCH EXPECTED OF THEM. I MEAN AS EXPERTS WHO RUN CLINICAL TRIALS OR STUDY BASE PARTICULAR DISEASES, THEY'RE EXPECTED TO UNDERSTAND THE LARGER PICTURE AS

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WELL. THEY MAY NOT SOLVE THE PROBLEM OF AFFORDABILITY, BUT IT BECOMES STANDARD OF CARE. THAT IS WHERE THIS LARGER COMES IN PLAY.

BUT IN TERMS OF ARTICULATING THE DEMOGRAPHIC, THE DISTRIBUTION, GEOSPATIAL DISTRIBUTION OF THESE PATIENTS AND THE BARRIERS THAT MAY EXIST, THAT'S A VERY REASONABLE EXPECTATION OF A RESEARCHER I WOULD SAY.

CHAIRPERSON BONNEVILLE: THANK YOU SO MUCH. AND I AGREE. SIMILAR COMMUNITY OUTREACH PLANS THAT WE STARTED TO REQUIRE OF OUR GRANTEES SORT OF FORCED EVERYONE TO THINK ABOUT THAT IN A DIFFERENT WAY OR MORE. SO TOO WILL ASKING SOMETHING LIKE THIS OF OUR RESEARCHERS. AND AS YOU MENTIONED, THEY MAY NOT SOLVE THE PROBLEM, BUT IT FORCES EVERYONE TO COALESCE AROUND A SPECIFIC ISSUE AND HOW TO SOLVE IT. SO THANK YOU.

DR. CREASEY: CAN I SAY SOMETHING?

CHAIRPERSON BONNEVILLE: I WANT -- ADRIENNE HAD HER HAND RAISED. NOW I SEE A THUMBS UP. I JUST WANT TO GO BACK AND SEE IF SHE HAS SOME COMMENTS BECAUSE I HAD FAILED TO RECOGNIZE HER. SO I APOLOGIZE.

MS. SHAPIRO: THAT'S OKAY. I JUST WANTED TO SAY THAT I ABSOLUTELY AGREE. THAT IS SOMETHING

THAT HAS BEEN CORE TO CIRM. IT'S PART OF US. I THINK IF THAT IS NOT KEPT IN THE RESEARCHER'S PATH, THAT WE CAN EASILY SLIP AWAY FROM ALL OF THE PROGRESS WE'VE MADE IN THE LAST TEN YEARS WITH THAT. SO IF THERE'S SOME WAY WE CAN ASSIST IN MAKING IT EASIER BY OUTLINING OR SUPPORTING HOW THEY CAN DO THAT, THAT MAY BE SOMETHING ON THE TABLE, BUT I DON'T THINK -- I THINK WE HAVE TO ABSOLUTELY REQUIRE IT OF THEM.

CHAIRPERSON BONNEVILLE: THANK YOU. ABLA, YOU WANTED TO MAKE ONE LAST COMMENT?

DR. CREASEY: I JUST WANTED TO SAY AGAIN, FOR THE INTEREST OF THE LAST THREE SPEAKERS, IS THAT THE FDA IS ACTUALLY REQUIRING WHAT YOU JUST DESCRIBED IN THE DESIGN OF THE CLINICAL TRIALS. SO IT'S NO LONGER DONE, BUT IT HAS TO ACCOMMODATE THE UNDERSERVED COMMUNITIES. THE GUIDANCE DOCUMENTS ALSO PROVIDE THAT INFORMATION. SO WE ARE GOING TO COMPLY WITH THAT AND ADD IT TO WHAT IS REQUIRED OF THE APPLICANTS TO EITHER CLIN2 OR CLIN4.

MS. SHAPIRO: THANK YOU.

CHAIRPERSON BONNEVILLE: THANK YOU. SO IF THERE ARE NO OTHER QUESTIONS OR COMMENTS, I PROPOSE WE MOVE INTO CLOSED SESSION. CAN YOU PLEASE READ THE LANGUAGE?

MR. AGUIRRE-SACASA: ABSOLUTELY. OKAY. WE'RE GOING TO ENTER INTO CLOSED SESSION FOR A DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO BLA STATUS FOR THE CLIN PORTFOLIO PURSUANT TO HEALTH AND SAFETY CODE 125290.30(F)(3) (B) AND (C). THANK YOU.

MARIVEL, ARE WE IN CLOSED SESSION?

MS. DE LA TORRE: EMILY IS GOING TO PUT US INTO CLOSED SESSION.

(THE WORKING GROUP THEN WENT INTO CLOSED SESSION, NOT REPORTED NOR HEREIN TRANSCRIBED. THE FOLLOWING WAS THEN HEARD IN OPEN SESSION:)

MR. AGUIRRE-SACASA: WE'RE BACK IN OPEN SESSION, AND I CAN ANNOUNCE THAT NO ACTION WAS TAKEN. GEOFF, I'M GOING TO TURN IT OVER TO YOU.

CHAIRPERSON BONNEVILLE: I THINK WE ARE -- UNFORTUNATELY I DO NOT BELIEVE WE'RE GOING TO HAVE TIME TO GO TO THE LAST TWO AGENDA ITEMS. GEOFF WILL REPORT BACK ON THAT IN AUGUST. AND I THINK THIS MEETING IS ADJOURNED. SO THANK YOU, EVERYONE, SO MUCH FOR PARTICIPATING TODAY. WE REALLY APPRECIATED ALL YOUR FEEDBACK.

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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 TREATMENT AND CURES ACCESSIBILITY AND AFFORDABILITY WORKING GROUP OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON MAY 14, 2024, 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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