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: JANUARY 17, 2024
8 A.M.

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

FILE NO.: 2024-03

I N D E X

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JANUARY 17, 2024; 8:00 A.M.

CHAIRMAN GOLDSTEIN: LET'S START WITH THE ROLL CALL PLEASE. IS THAT YOU, CLAUDETTE?

MS. MANDAC: I CAN. HAIFAA ABDULHAQ. MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MS. MANDAC: MONICA CARSON.

DR. CARSON: PRESENT.

MS. MANDAC: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: HERE.

MS. MANDAC: JUDY GASSON.

DR. GASSON: HERE.

MS. MANDAC: LARRY GOLDSTEIN.

CHAIRMAN GOLDSTEIN: HERE.

MS. MANDAC: DAVID HIGGINS.

DR. HIGGINS: PRESENT.

MS. MANDAC: VITO IMBASCIANI. PAT LEVITT.

DR. LEVITT: HERE.

MS. MANDAC: SHLOMO MELMED. CHRISTINE MIASKOWSKI.

DR. MIASKOWSKI: PRESENT.

MS. MANDAC: KAROL WATSON.

DR. WATSON: HERE.

MS. MANDAC: KEITH YAMAMOTO.

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MR. TOCHER: VITO STEPPED OUT. HE'S JUST COMING BACK IN, AND WE'LL HAVE A QUORUM.

CHAIRMAN GOLDSTEIN: WHY DON'T I GO AHEAD AND INTRODUCE THE MEETING. WE HAVE TWO MAJOR TOPICS TODAY. THE FIRST IS TO REVIEW COMMUNITY CARE CENTERS OF EXCELLENCE CONCEPT PLAN. AND THEN THE SECOND IS A TOPIC WITH WHICH I HAVE A CONFLICT OF INTEREST, AND SO IT WILL BE HANDLED BY SCOTT, WHO WILL THEN HAND IT OVER TO MARK FOR MANAGING THE DISCUSSION.

SO FIRST UP, COMMUNITY CARE CENTERS OF EXCELLENCE CONCEPT. WHO IS PRESENTING?

DR. LOMAX: THAT WOULD BE ME, GEOFF. LET ME PULL UP MY SCREEN. SO I DID WANT TO CHECK WITH THE WORKING GROUP. THE POSTED DECK, THIS IS A CONTINUATION EFFECTIVELY OF AN ITEM THAT WAS FIRST PRESENTED ON NOVEMBER 30TH. SO I DID -- THE FIRST SIX SLIDES OR SO ARE BACKGROUND ON THE LEAD-UP TO THE CONCEPT PLAN. AND I COULD FOREGO REPEATING THAT SET OF SLIDES IF IT WORKS FOR THE WORKING GROUP AND SORT OF DIVE INTO THE PROGRAMMATIC ELEMENTS, BUT I WANTED TO CHECK FIRST WITH THE WORKING GROUP TO SEE WHAT YOUR PLEASURE IS.

CHAIRMAN GOLDSTEIN: WHAT'S YOUR TIME ESTIMATE FOR THOSE SIX SLIDES, GEOFF?

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DR. LOMAX: IF I KIND OF SKIP THROUGH THEM QUICKLY, ABOUT SIX TO EIGHT MINUTES.

CHAIRMAN GOLDSTEIN: THEN I SUGGEST YOU DO THAT JUST TO BE SURE EVERYBODY IS LINED UP AND READY TO GO.

DR. LOMAX: OKAY. WILL DO. THANK YOU.

OKAY. SO THE MISSION STATEMENT: ACCELERATING WORLD-CLASS SCIENCE TO DELIVER TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND WORLD. I THINK THIS PARTICULAR INFRASTRUCTURE PROGRAM REALLY SPEAKS TO THE EQUITY AND DELIVERY ASPECTS OF THIS AS IT'S ABOUT EXTENDING OUR CLINICAL REACH BEYOND THE ALPHA CLINIC NETWORK.

I WANTED TO GIVE YOU A SENSE OF THE PROCESS THAT HAS LED UP TO THIS PLAN. STARTING IN OCTOBER 2022, WE INITIATED A NEEDS ASSESSMENT PHASE. THE NEEDS ASSESSMENT INITIALLY CONSISTED OF A SERIES OF LISTENING SESSIONS AT SITES INDICATED HERE, IN THE CENTRAL VALLEY, INLAND EMPIRE, AND COACHELLA VALLEY. THESE LISTENING SESSIONS WERE ATTENDED BY A RANGE OF PARTICIPANTS THAT HAD EXPERIENCE BOTH IN OPERATING CLINICAL CENTERS, INDIVIDUALS INVOLVED IN HEALTH EDUCATION, HEALTH NAVIGATION, COMMUNITY HEALTH WORKERS, COMMUNITY GROUPS, RESEARCHERS, A

VERY DIVERSE RANGE OF PARTICIPANTS THAT HAVE INTEREST IN THE PROGRAMMATIC ASPECTS OF THIS INITIATIVE AND POTENTIALLY REPRESENTS INDIVIDUALS THAT MAY APPLY ONCE THE APPLICATION BECOMES LIVE.

WE THEN IN JUNE OF 2023 SPONSORED A STATEWIDE PUBLIC WORKSHOP. A NUMBER OF YOU PARTICIPATED IN THAT PROGRAM. THE AIM OF THE WORKSHOP WAS TO PULL TOGETHER OUR FINDINGS FROM THE NEEDS ASSESSMENT AND GET FEEDBACK, AGAIN, FROM A SIMILARLY DIVERSE POOL OF STAKEHOLDERS IN TERMS OF IDEAS FOR PROGRAM DIRECTION. AND COMING OUT OF THAT WORKSHOP, WE THEN DEVELOPED THE DRAFT CONCEPT PLAN BASED ON, IN PART, WHAT WE HEARD IN THE WORKSHOP.

THAT CONCEPT PLAN HAS BEEN GOING THROUGH A REVIEW PROCESS THAT INCLUDES THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP. MEMBERS OF THIS SUBCOMMITTEE HAVE PROVIDED COMMENTS AND FEEDBACK, WHICH I'LL ADDRESS LATER WHEN I HIT ON THE PROGRAMMATIC ASPECTS. AND ACTUALLY YESTERDAY WE PRESENTED PARTICULARLY COMMUNITY ENGAGEMENT ASPECTS TO THE MEDICAL AND ETHICAL STANDARDS WORKING GROUP TO EXPLORE, PARTICULARLY AROUND IMPLEMENTATION, BEST PRACTICE, ETHICAL PRACTICE, WAYS IN WHICH COMMUNITY ENGAGEMENT COULD OCCUR IN CONCORDANCE WITH HIGH MEDICAL AND ETHICAL STANDARDS WE EXPECT AT CIRM.

PROVIDED YOU ALL ARE SUPPORTIVE OF THIS PLAN AND THE BOARD, WE WOULD THEN ENTER AN APPLICATION PHASE LATER THIS YEAR.

THE AIM OF THE PROGRAM IS SPELLED OUT TO SOME EXTENT IN PROPOSITION 14. IT'S TO EXPAND THE ALPHA STEM CELL CLINIC PROGRAM, PROMOTE HUMAN CLINICAL TRIALS, AND ACCESS TO THOSE TRIALS IN GEOGRAPHICALLY DIVERSE REGIONS OF THE STATE. AND THE AIM WOULD BE BOTH TO MAKE CLINICAL RESEARCH AVAILABLE TO RESIDENTS AND SUBSEQUENT CURES THAT EMERGE FROM CIRM RESEARCH.

IN TERMS OF INFRASTRUCTURE PROGRAMS, ONE OF THE SORT OF QUESTIONS THAT ACTUALLY CAME UP QUITE A BIT ALONG THE PROCESS IS WHAT DO THESE LOOK LIKE. OFTEN WHEN YOU SAY A CENTER, ONE ENVISIONS LARGE BUILDINGS AND SHINY OBJECTS. OUR INFRASTRUCTURE PROGRAM TYPICALLY, WHILE THEY INVOLVE THESE BUILDINGS, WILL BE FUNDING OUR PEOPLE WHO MAINTAIN THE OPERATIONS. AND TO GIVE YOU AN IDEA, THIS IS A TYPICAL TEAM THAT WOULD BE REQUIRED TO DELIVER CELL AND GENE THERAPIES TO PATIENTS AT A MEDICAL CENTER.

AND THIS REALLY REFLECTS SORT OF THE BUDGET OF AN ALPHA CLINIC. THESE BUDGETS INCLUDE INDIVIDUALS WHO CAN ENGAGE SPONSORS WHO ARE BRINGING TRIALS INTO THESE SITES, PATIENT EDUCATORS,

NAVIGATORS, AND RESEARCH COORDINATORS THAT COULD DO EVERYTHING FROM INTRODUCE PATIENTS TO THE TRIAL, CONDUCT THE INFORMED CONSENT, AND THEN FOLLOW THE PATIENT THROUGH THEIR CLINICAL COURSE, INDIVIDUALS WHO PERFORM REGULATORY AND COVERAGE ANALYSIS TO DEAL WITH THE FINANCIAL AND BILLING ASPECTS OF THE TRIAL. THEN WE ALSO TYPICALLY SEE SOME SORT OF SUPPORT FOR PRODUCT MANAGEMENT, PHARMACY, AND MANUFACTURING, ALTHOUGH THE MAJOR PIECES OF THE MANUFACTURING ASPECTS WOULD POTENTIALLY BE HANDLED THROUGH OUR MANUFACTURING INITIATIVE, BUT IT'S A VERY -- THAT'S A TOUCHPOINT BETWEEN OUR DIFFERENT INFRASTRUCTURE PROGRAMS WITH MANUFACTURING AND CLINICAL. AND THERE'S STRONG DATA MANAGEMENT PIECES, FOR EXAMPLE, LOOKING AT PATIENT REGISTRIES TO HELP DEVELOP COHORTS FOR THESE TRIALS.

SO WHEN WE LOOK AT -- THIS IS -- NOW, THE AIM IS TO PROVIDE SORT OF THE BIG PICTURE OF HOW OUR DIFFERENT INFRASTRUCTURE AND SUPPORT PROGRAMS WOULD COME TOGETHER TO SUPPORT PATIENTS. THE AIM OF THIS GRAPHIC IS TO REALLY ILLUSTRATE THE STRATEGIC FOCUS TO PUT THE PATIENTS IN THE CENTER OF A SUPPORT SYSTEM. AND THAT SYSTEM CURRENTLY INCLUDES THE ALPHA CLINICS, WHICH I HAVE BEEN REFERRING TO. THERE ARE NINE AWARDS, TEN SITES, AND THEY'RE

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SUPPORTING ALMOST ALL THE CIRM-FUNDED CLINICAL TRIALS. THOSE CLINICAL TRIALS, 96 OF WHICH HAVE BEEN FUNDED TO DATE, AS YOU ARE WELL AWARE, WERE FUNDED THROUGH OUR CLINICAL STAGE PROGRAMS. SO WE'VE KIND OF GOT AN AXIS HERE WHERE WE HAVE THOSE CLINICAL TRIALS UP AND INITIATED AT SITES THAT ARE QUITE CAPABLE OF DELIVERING THOSE TREATMENTS. AND THE AIM IS TO FURTHER EXPAND THAT INFRASTRUCTURE TO SUPPORT ADDITIONAL PATIENTS COMING INTO THOSE TRIALS.

AGAIN, ANOTHER PROGRAM THAT IS NOW PENDING REVIEW BY THE GRANTS WORKING GROUP AND ACCESS AND AFFORDABILITY WORKING GROUP IS OUR PATIENT SUPPORT PROGRAM AIMED AT ADDRESSING FINANCIAL AND LOGISTICAL BARRIERS. THAT'S GOING TO BE AN IMPORTANT PIECE OF THIS PICTURE BECAUSE THAT PROGRAM WILL BE ABLE TO SUPPORT PATIENTS, PARTICULARLY THOSE, AGAIN, WITH FINANCIAL AND LOGISTICAL NEEDS, WHICH, BASED ON OUR NEEDS ASSESSMENT FINDINGS, THAT WAS A STRONG EMPHASIS OF PARTICIPANTS, THAT IN A LOT OF THESE REGIONS, THERE ARE CONSIDERABLE CHALLENGES WITH TRAVEL, CHILDCARE, AND THE RANGE OF DEMANDS THAT ARE PUT ON PATIENTS IN ORDER TO PARTICIPATE IN THESE TRIALS.

AGAIN, HERE THE COMMUNITY CARE CENTERS OF

EXCELLENCE, WHICH IS TO PROVIDE THAT PHYSICAL INFRASTRUCTURE FOR THESE CLINICAL PROGRAMS SO THAT THE DEMANDS I ALLUDED TO EARLIER ADDRESSED THROUGH THE PATIENT SUPPORT PROGRAM ARE REDUCED OR ATTENUATED BECAUSE CERTAIN ASPECTS OF THE TRIAL OR THE ENTIRETY OF THE TRIAL CAN BE DELIVERED TO THE PATIENT IN GREATER PROXIMITY WITH THE GREATER COMMUNITY SUPPORT, LESS TRAVEL, LESS DEMAND. SO THAT'S THE OVERALL CONCEPT LEVEL WHERE WE'RE GOING WITH THIS ONE.

SO I'M NOW GOING TO GET INTO THE MAJOR COMPONENTS OF THE CONCEPT PLAN THAT YOU HAVE BEFORE YOU. AS WITH ANY CLINICAL INFRASTRUCTURE, BECAUSE WE'RE TALKING ABOUT CLINICAL TRIAL, THERE'S A CLINICAL COMPONENT TO THIS. THAT PIECE IS BROKEN INTO TWO PARTS. I'LL ELABORATE IN A BIT MORE DETAIL IN SUBSEQUENT SLIDES. IT'S TO SUPPORT OR CONDUCT CLINICAL TRIALS. SO THERE'S TWO PIECES TO THAT OPTION A OR OPTION B. AND, AGAIN, I'LL ELABORATE.

AND ULTIMATELY ALSO POSITION THESE CENTERS TO DELIVER APPROVED REGENERATIVE MEDICINE PRODUCTS. AND IN SOME CASES WHERE THOSE SITES MAY NOT BE POSITIONED IN THE AWARD PERIOD TO SERVE AS A DELIVERY SITE FOR THESE PRODUCTS OR THESE CLINICAL TRIALS, TO THEN SERVE AS A REFERRAL HUB FOR ALPHA

CLINICS PATIENTS WHERE THEY COULD RECEIVE TREATMENT AT, SAY, AN ALPHA CLINIC SITE, BUT COME BACK TO THE CENTER FOR FOLLOW-UP AND, AGAIN, TRYING TO REDUCE THE OVERALL DEMANDS OF THE TRIAL.

ALSO, THERE'S A CAREER DEVELOPMENT PROGRAM EMBEDDED IN THE CONCEPT PLAN. AND THE AIM HERE IS REALLY TO ADAPT AND DEPLOY TRAINING CURRICULA THAT ALREADY EXISTS OR ALREADY BEEN DEVELOPED THROUGH, AGAIN, THE ALPHA CLINICS, CIRM EDUCATION PROGRAMS, AND TO REALLY SERVE AS A PLACEMENT SITE FOR CIRM TRAINEES.

I THINK ONE OF THE MOST SORT OF NICEST COMMENTS I PICKED FROM THE NEEDS ASSESSMENT WERE A NUMBER OF THE ALPHA CLINIC DIRECTORS REALLY INDICATING THERE'S A STRONG APPETITE CERTAINLY FOR CLINICAL FELLOWS TO BE ABLE TO BE PLACED IN CENTERS THAT ARE REALLY OUTSIDE SOME OF THESE MAJOR ACADEMIC CENTERS. THERE SEEMS TO BE A STRONG INTEREST THERE. I THINK IT DERIVES FROM A COMMITMENT TO DIVERSITY AND INCLUSION IN CLINICAL RESEARCH. CERTAINLY WAS THE MESSAGE FROM THE DIRECTORS THAT WE'RE FUNDING ALREADY.

FINALLY, I THINK THE PIECE THAT'S PARTICULARLY UNIQUE TO THIS PROGRAM ARE THE COMMUNITY ENGAGEMENT ASPECTS. THE IDEA IS TO ENGAGE

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WITH PATIENTS IN COMMUNITIES AND PARTICULARLY TRYING TO LEVERAGE COMMUNITY-BASED ORGANIZATIONS. THAT'S BEEN AN EXTREMELY STRONG MESSAGE THAT WE'VE RECEIVED CONSISTENTLY OVER THE COURSE OF BOTH THE NEEDS ASSESSMENT, SUBSEQUENT MEETINGS, AGAIN RESURFACED AT THE STANDARDS WORKING GROUP MEETING YESTERDAY, THAT FOR CERTAIN POPULATIONS THAT WE HAVE COMMITTED TO PROVIDE ACCESS TO, THERE'S A LEVEL OF TRUST AND ENGAGEMENT THAT, IN ORDER TO SUCCESSFULLY ACHIEVE THOSE OBJECTIVES, THERE'S A COMPELLING CASE THAT THAT ONLY HAPPENS WITH SUPPORT AT THE COMMUNITY LEVEL, COMMUNITY SUPPORT STRUCTURES, WHETHER IT BE FAITH-BASED ORGANIZATIONS OR COMMUNITY-BASED CENTERS, WHERE INDIVIDUALS TURN FOR ADVICE AND SUPPORT. WE HAVE TO BRING SOME OF THOSE ENTITIES IN IN ORDER TO REALLY CAPTURE THAT POPULATION. AND THAT'S, AGAIN, THE FOCUS PARTICULARLY ON THOSE UNDERREPRESENTED POPULATIONS. THEY'RE UNDERREPRESENTED FOR A REASON. OUR HYPOTHESIS IS THAT FUNDING AT THIS LEVEL WILL HELP ADDRESS THAT.

SO I WANT TO DIG A LITTLE BIT DEEPER INTO THE CORE ELIGIBILITY REQUIREMENTS AND SOME OF THESE ACTIVITIES IN PART BECAUSE THEY ADDRESS QUESTIONS THAT YOU ALL RAISED -- HAVE RAISED PREVIOUSLY. SO THIS GETS TO ISSUES OF QUALITY, ASSURANCE, SAFETY,

ETHICS.

SO IN TERMS OF CLINICAL OPERATIONS, TO BE ELIGIBLE A FACILITY WOULD HAVE TO BE A LICENSED HEALTHCARE FACILITY WITH A DEMONSTRATED CAPACITY TO SUPPORT HUMAN SUBJECTS PROTOCOLS IN A HEALTH RESEARCH CONTEXT. THAT'S STILL FAIRLY BROAD, BUT IT MEANS THEY'RE REALLY ABLE TO CONDUCT RESEARCH UNDER THE OVERSIGHT STRUCTURES THAT WE HAVE ADOPTED TO GUIDE ALL OF OUR RESEARCH THAT INVOLVES HUMAN SUBJECTS. AND HAVE THE CAPACITY TO BE IN THE PROCESS OF DEVELOPING THE CAPACITY TO SUPPORT CLINICAL PROTOCOLS INVOLVING CELL AND GENE THERAPY OR REGENERATIVE MEDICINE. AND THEY DO NOT PRACTICE THE ADMINISTRATION OF UNAUTHORIZED STEM CELL TREATMENTS, AND I'LL COME BACK IN A MOMENT.

CAREER DEVELOPMENT, HAVE A DEMONSTRATED CAPACITY TO SUPPORT EDUCATION, TRAINING, CAREER DEVELOPMENT OF PHYSICIANS, NURSES, COMMUNITY COORDINATORS, COMMUNITY HEALTH WORKERS, AND OTHER HEALTH AND MEDICAL PROFESSIONALS. AGAIN, ON THE OUTREACH AND ENGAGEMENT SIDE, HAVE A TRACK RECORD OF CONDUCTING THIS TYPE OF WORK. AGAIN, THE ORGANIZATIONS THAT WE ENGAGE, THE CLINICAL CENTERS, ARE ALREADY CONDUCTING A LOT OF THESE ACTIVITIES IN DIFFERENT DOMAINS, NOT NECESSARILY REGENERATIVE

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MEDICINE. SO THIS IS, I THINK, A STANDARD OF PRACTICE WITHIN THE COMMUNITY MEDICAL CENTERS. AND THE AIM HERE WOULD BE TO EXPAND THAT EXPERTISE TO REGENERATIVE MEDICINE AND CELL AND GENE THERAPY.

SO I ALLUDED TO THIS EARLIER. I WANTED TO SORT OF COME BACK TO HOW WE THINK THE CLINICAL OPERATION, HOW WE'RE PROPOSING THE CLINICAL OPERATIONS SIDE IN THE CONCEPT PLAN. AND THIS REALLY REFLECTS WHAT WE SAW IN TERMS OF CAPACITY AND READINESS OF SITES. THERE WAS A DIVIDE OUT THERE. THERE ARE CERTAINLY SITES THAT ARE CAPABLE AND THEY'RE ACTUALLY ALREADY SUPPORTING ACCESS TO REGENERATIVE MEDICINE CLINICAL TRIALS. PARTICULARLY IN ONCOLOGY, WE HAVE A NUMBER OF SITES THAT PROVIDE REFERRALS TO PATIENTS THAT ULTIMATELY GET TREATED AT A LOT -- A NUMBER OF WHICH GET TREATED AT ALPHA CLINICS. SO THAT REFERRAL NETWORK EXISTS ALREADY.

BUT A NUMBER OF THOSE SITES DON'T SEE THEMSELVES IN THE POSITION OVER THE AWARD PERIOD OF DEVELOPING THE CAPACITY TO DELIVER REGENERATIVE MEDICINE PRODUCTS. IT MIGHT BE A LONGER TERM AIM, BUT THEY DON'T NECESSARILY SEE THEMSELVES IN A POSITION TO GET TO THAT POINT IN THE NEXT FIVE YEARS.

IN CONTRAST, THERE ARE SITES THAT ARE IN

THE PROCESS OF DEVELOPING THE CAPACITY TO EITHER HANDLE MANUFACTURED PRODUCTS, DEVELOP GMP FACILITIES THAT REALLY COULD SUPPORT AND DELIVER TRIALS. SO WE CREATED TWO OPTIONS THERE. AND THESE OPTIONS, WHEN WE GET TO SORT OF SOME OF THE BUDGET ISSUES, THERE WOULD BE A DIFFERENTIAL BUDGET DEPENDING ON HOW THE APPLICANT ORGANIZATION WANTED TO COME IN THERE.

CAREER DEVELOPMENT, ONE OF THE PARTS THERE IS WE ARE REALLY LOOKING FOR THEM TO ADAPT OR OTHERWISE UTILIZE EDUCATION AND CIRM TRAINING RESOURCES. AT THE LAST BOARD MEETING, YOU WERE PROVIDED WITH A DESCRIPTION OF THE EDUCATION PORTAL WHICH IS CURRENTLY UNDER DEVELOPMENT. WE VIEW THOSE SORT OF CROSS-CUTTING PLATFORMS AS TECHNOLOGIES THAT WOULD BE INTEGRATED INTO THIS AWARD FROM THE STANDPOINT OF MAKING THEIR TRAINING OPTIONS VISIBLE ON THESE PLATFORMS, MAKING POSITIONS AND PLACEMENT OPPORTUNITIES VISIBLE TO OTHER CIRM TRAINEES. SO IT GOES PART AND PARCEL WITH SOME OF THE RESOURCES THAT ARE BEING DEVELOPED INTERNALLY AT CIRM.

AND ENGAGEMENT AND OUTREACH EXTEND THESE PROGRAMS PARTICULARLY -- AND, AGAIN, I WANT TO EMPHASIZE PARTICULARLY THE ALPHA CLINICS ARE REALLY TAKING THE LEAD AND DEVELOPING COMMUNITY ENGAGEMENT CAPACITY, RESOURCES, AND PROGRAM. I THINK THE NEXT

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STEP IS TO THEN EXTEND THE REACH OF SOME OF THESE PROGRAMS BY BUILDING PARTNERSHIPS WITH COMMUNITY-BASED ORGANIZATIONS TO SUPPORT THE AIMS OF THIS CONCEPT PLAN.

A LITTLE BIT OF A COMPARE AND CONTRAST HERE BECAUSE THESE ARE NOT NECESSARILY MINI ALPHA CLINICS, ALTHOUGH PERHAPS THEY COULD BE, BUT THE ALPHA CLINICS CHARGE IS REALLY TO CONDUCT CIRM CLIN2 ELIGIBLE TRIALS. THAT WAS -- FROM A CLINICAL STANDPOINT, THAT WAS THE ELIGIBILITY CRITERIA. ON THE COMMUNITY CARE SIDE, IT'S THE CAPACITY TO SUPPORT THOSE PROGRAMS, AGAIN, POTENTIALLY SERVING AS A REFERRAL SITE OR DEVELOP THE CAPACITY TO SUPPORT CLIN2 ELIGIBLE TRIALS. THEY MAY NOT HAVE IT ON DAY ONE, BUT THEY WOULD DEVELOP THAT CAPACITY OVER THE AWARD PERIOD, AGAIN, IN CONTRAST TO THE ALPHA CLINICS THAT HAD TO HAVE THAT CAPACITY AS A CONDITION OF APPLICATION.

THE ALPHA CLINICS REALLY HAVE LED THE WAY IN DEVELOPING CLINICAL TRAINING PROGRAMS. AGAIN, THE COMMUNITY CARE CENTERS WOULD BE APPLYING THESE PROGRAMS AND SERVING AS PLACEMENT SITES FOR TRAINEES IN THE ALPHA CLINICS OR IN OTHER SETTINGS.

AND THE ENGAGEMENT ACTIVITIES, FOR THE MOST PART, OF THE ALPHA CLINICS, ALTHOUGH THIS IS

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EVOLVING OVER THE LIFE OF THESE AWARDS, IS FOCUSED PREDOMINANTLY ON CLINICAL TRIAL ENGAGEMENT AND NAVIGATION AND MEETING WITH PATIENT GROUPS; WHEREAS, IN THE CONTEXT OF THE COMMUNITY CARE CENTERS, THERE WOULD BE CERTAINLY THAT ENGAGEMENT AND NAVIGATION IN CLINICAL TRIALS, BUT ALSO BROADER ENGAGEMENT AROUND CERTAIN ISSUES. FOR EXAMPLE, ONE OF THE ONES THAT COMES UP QUITE FREQUENTLY IS HELPING COMMUNITY MEMBERS REALLY UNDERSTAND THE DIFFERENCE BETWEEN CLINICAL RESEARCH, CLINICAL TRIALS THAT ARE AIMED AT REALLY HELPING PATIENTS AND SOME OF THE TREATMENTS OUT THERE THAT REALLY PRESENT FINANCIAL OR MEDICAL TOXICITY TO PATIENTS. SO A BIT OF A BROADER RANGE OF ACTIVITIES, SOME OF WHICH SUPPORT REGENERATIVE MEDICINE, BUT MAY NOT BE CARRIED OUT IN THE CONTEXT OF A SPECIFIC CLINICAL PROTOCOL.

AGAIN, COMING BACK TO SOME OF THE QUESTIONS THAT YOU ALL HAVE POSED THAT DESERVE SOME ELABORATION BASED ON YOUR FEEDBACK. THERE WAS SORT OF HOW -- I THINK THE QUESTION WAS AROUND SORT OF HOW WE MAINTAIN THE ETHICS OF THE STANDARDS HERE. AGAIN, I'VE ALLUDED TO THE FACT THAT EXPERIENCE WITH HUMAN SUBJECT PROTOCOLS AND IRB OVERSIGHT WILL BE A CONDITION OF APPLICATION. I ALLUDED TO THIS EARLIER, UNAUTHORIZED STEM CELL TREATMENTS. THERE'S

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ALREADY EXISTING STATUTE IN CALIFORNIA THAT REQUIRES DISCLOSURE TO PATIENTS IF YOU ARE PROVIDING A DIRECT FEE-FOR-SERVICE TREATMENT INVOLVING, QUOTE, UNQUOTE, STEM CELL THERAPIES. AND THAT'S DEFINED UNDER THE STATUTE. AND PARTICULARLY -- WHAT THIS IS AIMED AT DOING IS ALERTING PATIENTS TO TREATMENTS THAT HAVE NOT BEEN FDA AUTHORIZED. AND FDA AUTHORIZATION HAS BEEN A CONDITION OF ALL CIRM CLINICAL PROGRAMS. SO IF YOU'RE PROVIDING THIS WARNING, PLEASE DON'T BOTHER APPLYING TO THIS PROGRAM.

RESEARCH ETHICS TRAINING IS SOMETHING, AGAIN, WE SORT OF -- IT'S KIND OF COME UP SUBSEQUENT TO YOUR INITIAL INQUIRY. AND WE'VE GONE OUT AND REALLY LOOKED AT PARTICULARLY WHAT ARE THE TRAINING AND CERTIFICATION OPPORTUNITIES FOR PEOPLE THAT ARE DOING COMMUNITY ENGAGEMENT MORE GENERALLY AROUND IN THIS SPACE, AGAIN, AS OPPOSED TO NAVIGATING WITHIN A SPECIFIC CLINICAL TRIAL PROTOCOL, WHICH WOULD BE COVERED UNDER THE IRB. THERE'S A BROADER SET OF TRAINING OPPORTUNITIES THAT ARE ESSENTIALLY BEST PRACTICES IN THE FIELD FOR NAVIGATORS, COMMUNITY HEALTH WORKERS. AND THE PROPOSAL WOULD BE TO PROVIDE FUNDING OPPORTUNITIES TO SUPPORT THE TRAINING AND ACCREDITATION OF ANYONE IN THIS PROGRAM TO ACQUIRE THAT LEVEL OF TRAINING THAT IS BECOMING A

STANDARD OF PRACTICE FOR THE FIELD.

AND AS I MENTIONED, THE STANDARDS WORKING GROUP ALSO REVIEWED THE CONCEPT PLAN. WE HAD A DISCUSSION AROUND THE PATIENT ENGAGEMENT ASPECTS OF THE CONCEPT PLAN. AND THEY'VE GIVEN US A NUMBER OF CONCRETE RECOMMENDATIONS FOR STEPS THAT CAN BE TAKEN AT THE APPLICATION LEVEL TO, AGAIN, FOCUS ON ISSUES LIKE BOTH THE TRAINING AND ACCREDITATION, BUT HOW WILL YOU REIMBURSE ADVISORS, COMMUNITY INDIVIDUALS WHO ARE SUPPORTING THESE PROGRAMS. SO A RANGE OF OPERATIONAL RECOMMENDATIONS THAT REALLY HELP BUILD UP A RESPONSIBLE PROGRAM THAT WE THINK WILL MEET CIRM STANDARDS.

AND THEN A COUPLE OF OTHER PIECES THAT, AGAIN, IN RESPONSE TO ISSUES THAT I THINK WERE -- THAT CAME FROM BOTH THE ACCESS AND AFFORDABILITY WORKING GROUP AND YOURSELVES WAS -- I THINK THE QUESTION CAME UP IN TERMS OF OFTEN PARTNERSHIPS ARE PROPOSED, BUT HOW DO WE KNOW THOSE DOLLARS WILL ACTUALLY FLOW TO, SAY, THOSE GROUPS. WE PROPOSE THAT THE BUDGET WOULD -- THE BUDGET LINE ITEM FOR THESE COMMUNITY-BASED PARTNERSHIPS WOULD BE A SEPARATE LINE ITEM DEDICATED TO THAT PURPOSE SO THAT THOSE FUNDS WOULD FLOW ONCE THOSE PARTNERSHIP AGREEMENTS WERE IN PLACE.

THERE WERE QUESTIONS OF SUSTAINABILITY. AND WE'VE BEEN LOOKING AT A NUMBER OF WAYS IN WHICH THE, AGAIN, PARTICULARLY THE NAVIGATION, EDUCATION, AND COMMUNITY ENGAGEMENT PIECES CAN TIE IN TO ESTABLISHED REIMBURSEMENT PROGRAMS. AND TWO VERY PROMISING PROGRAMS WHERE WE CAN TRAIN PEOPLE UP AND ALLOW THEM TO BE ELIGIBLE FOR REIMBURSEMENT IS THE COMMUNITY HEALTH WORKER CERTIFICATE PROGRAM AND PATIENT NAVIGATION CERTIFICATE PROGRAM. AND WE'VE SPOKEN TO A NUMBER OF ORGANIZATIONS THAT ACTUALLY PROVIDE THESE CERTIFICATIONS AND TRAINING. AND THEY'RE AWARE OF THIS PROGRAM AND WOULD BE VERY INTERESTED IN EXPLORING HOW THEY COULD PARTNER IN.

BUILDING COMPETENCY, I THINK ONE OF THE POINTS WAS HOW DO WE HELP DEVELOP BEST PRACTICES AS OPPOSED TO LETTING A THOUSAND FLOWERS BLOOM AND NOT KNOWING WHAT THE RIGHT ANSWER IS. I THINK THE POINT HERE IS THAT CIRM NEEDS TO HELP MEDIATE SOME OF THESE ENGAGEMENT ACTIVITIES. WE CAN PROVIDE CONTENT AREA EXPERTISE, WE CAN LEVERAGE EXPERTISE THAT'S BEING DEVELOPED IN THE ALPHA CLINICS NETWORK. FOR EXAMPLE, THE ALPHA CLINICS NOW IS FORMING A WORKING GROUP TO WORK ON ENGAGEMENT RESOURCES, IN PARTICULAR EDUCATION MATERIALS. SO WE REALLY SEE AN ACTIVE MANAGEMENT ROLE THAT THE CIRM TEAM CAN PLAY

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SPECIFICALLY IN MEDIATING THIS SORT OF PIECE WHERE WE'RE DEVELOPING CONTENT AND MATERIALS AND MAKING SURE THEY'RE BEING EVALUATED AND THEY'RE APPROPRIATE IN TERMS OF THE INFORMATION THEY'RE PROVIDING, THE MESSAGING, ET CETERA.

AND I THINK WHAT I'VE SUGGESTED AND, AGAIN, IN CONSULTATION WITH YOU ALL IS THAT THE MEDICAL AFFAIRS TEAM PROPOSES TO BRING IN CAPACITY TO OUR TEAM WITH EXPERTISE IN BOTH PROGRAM PLANNING AND EVALUATION IN A QUANTITATIVE WAY TO REALLY FIELD TEST AND EVALUATE THOSE ACTIVITIES TO PROVIDE YOU WITH METRICS OF PROGRAM EFFECTIVENESS.

AND I THINK WE COVERED THIS LAST TIME. I DON'T WANT TO GO INTO A LOT OF DETAIL HERE. I JUST WANT TO FINISH. SO, AGAIN, THIS WOULD HAPPEN IN PARTNERSHIPS WITH THE ALPHA CLINICS. AND THE ALPHA CLINICS HAVE BEEN ON BOARD THROUGHOUT THE PROCESS. AND THE MANUFACTURING NETWORK IS ALSO STARTING TO HAVE CONVERSATIONS THAT WOULD TIE IN PARTICULARLY TO CENTERS THAT WANT TO DEVELOP MANUFACTURING CAPACITY.

I MENTIONED HOW WE'RE COORDINATING WITH THE EDUCATION PORTAL. AND WE HAVE REACHED OUT TO A NUMBER OF RARE DISEASE GROUPS, ONE OF WHICH PRESENTED YESTERDAY AT THE STANDARDS GROUP, TO HELP UNDERSTAND BEST PRACTICES IN TERMS OF ENGAGING THOSE

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PATIENTS.

THIS IS DIRECTLY FROM THE CONCEPT PLAN. AGAIN, A BUDGET ALLOCATION PROPOSED OF 60.2 MILLION, AGAIN, TO SUPPORT CORE OPERATIONS, COMMUNITY PARTNERSHIPS. AND THERE IS EQUIPMENT -- THERE ARE FACILITIES FUNDS IN THIS AWARD, AND WE WOULD HAVE -- THERE IS AN OPPORTUNITY FOR SITES, PARTICULARLY THOSE WANTING TO DEVELOP THE CAPACITY TO DELIVER TREATMENTS, TO APPLY FOR FACILITIES FUNDING.

AND IF YOU -- WE ARE JUST PROJECTING THIS OUT IN TERMS OF THE LANDSCAPE I'VE DESCRIBED. THIS BUDGET CONCEIVABLY COULD SUPPORT THREE SITES THAT WOULD APPLY TO BOTH SUPPORT AND DELIVER CLINICAL TRIALS AT ROUGHLY A LEVEL OF ABOUT 10 MILLION PER YEAR. THAT'S CONSISTENT WITH AN ALPHA CLINIC AWARD. AND SUPPORT AWARDS COMING IN AT ABOUT 25 PERCENT LESS, AT ABOUT 7.5 MILLION PER YEAR, MAINLY BECAUSE OF REDUCED STAFF DEMANDS AROUND THE CLINICAL TRIAL SUPPORT.

I THINK WITH THAT, I WILL PUT FORWARD OUR REQUEST THAT YOU APPROVE THIS CONCEPT PLAN AND OPEN IT UP FOR DISCUSSION.

CHAIRMAN GOLDSTEIN: GREAT. SO LET ME LEAD THIS OFF, GEOFF. WHO DO YOU IMAGINE WOULD BE PI ON ONE OF THESE APPLICATIONS? AND WHAT SORT OF

GOVERNANCE STRUCTURE DO YOU IMAGINE FOR THESE ORGANIZATIONS?

DR. LOMAX: SO TYPICALLY IN A CLINICAL, AGAIN, ALPHA CLINICS BEING THE MODEL, FUNDAMENTALLY THERE'S STILL A CLINICAL COMPONENT TO THESE AWARDS. AND TRADITIONALLY IT'S BEEN A LICENSED MEDICAL DIRECTOR LEVEL INDIVIDUAL AT THE APPLICANT INSTITUTION. I THINK THAT'S IMPORTANT FOR A NUMBER OF REASONS. FIRST OF ALL, BECAUSE WE'RE DEALING WITH THE PRACTICE OF MEDICINE ULTIMATELY IN CLINICAL RESEARCH, THAT LEVEL OF CREDENTIAL IS ALMOST ESSENTIAL. BUT ALSO, AGAIN, AT THE DIRECTOR LEVEL, WE FIND THAT THOSE INDIVIDUALS ARE AN AGENCY WITHIN THE APPLICANT ORGANIZATION ITSELF. AND I THINK THAT AGENCY IS REALLY CRITICAL TO MOVING THESE PROGRAMS. THIS FUNDING REQUIRES AN INDIVIDUAL WHO CAN NAVIGATE AND WORK, SAY, WITH A CANCER CENTER, WHICH THERE MAY BE SOME PAIN INITIALLY COMING IN BECAUSE CANCER CENTERS ARE WELL ESTABLISHED AND THEY ALSO TOUCH ON REGENERATIVE MEDICINE TREATMENTS.

SO I THINK IT'S THAT SOMEONE AT THAT LEVEL WITH THAT, AGAIN, CLINICAL CREDENTIALS, BUT ALSO AT A LEVEL WITHIN THE APPLICANT ORGANIZATION TO SUCCESSFULLY IMPLEMENT THE PROGRAM.

CHAIRMAN GOLDSTEIN: FOR GOVERNANCE DO YOU

IMAGINE SOME SORT OF STEERING COMMITTEE COMPOSED OF --

DR. LOMAX: SORRY. SO WE HAVE HAD A VERY SUCCESSFUL TRACK RECORD OF USING STEERING COMMITTEE-TYPE PROCESSES ACROSS OUR INFRASTRUCTURE PROGRAMS. IT'S CERTAINLY A MODEL THAT -- MY RECOMMENDATION WOULD BE WE REPLICATE THAT IN THE CONTEXT OF THIS AWARD. I THINK THE QUESTION BECOMES, AND AGAIN THIS GOES BACK TO THE LANGUAGE OF PROPOSITION 14, THERE'S A CONNECTION OR A COLLABORATION WITHIN THE ALPHA CLINICS THAT'S SORT OF, I WON'T SAY IMPLIED, BUT SUGGESTED BY THE PROPOSITION. AND THE ALPHA CLINICS HAVE ALREADY BEEN -- A NUMBER OF SITES HAVE ALREADY BEEN WORKING WITH POTENTIAL APPLICANTS TO START SOME OF THESE PROCESSES. SO I THINK THE QUESTION BECOMES, BEYOND THE SORT OF PROGRAMS IN THIS AWARD, HOW THAT RELATIONSHIP THEN MAY OR MAY NOT CONNECT UP WITH, SAY, THE ALPHA CLINICS IS AN OPEN QUESTION THAT'S WORTH EXPLORING.

CHAIRMAN GOLDSTEIN: QUESTIONS FROM OTHER MEMBERS OF THE SUBCOMMITTEE?

MS. MANDAC: PAT AND THEN CHRIS HAD THEIR HANDS RAISED.

DR. LEVITT: THANKS, GEOFF.

TWO THINGS I WANTED TO EMPHASIZE. GEOFF AND I HAVE HAD CONVERSATIONS OVER A SUPPOSED BREAK, I GUESS, IN ACTIVITY DURING THE HOLIDAYS. AND THERE'S SEVERAL THINGS THAT I BROUGHT UP. ONE WAS THAT THE PROGRAM IS HIGHLY DEPENDENT UPON REFERRALS. AND THE PATIENT NAVIGATORS TYPICALLY HAVE THE JOB OF FACILITATING THE ABILITY OF A REFERRED PATIENT TO GET TO THE CLINIC TO PARTICIPATE IN ORDER TO GET CLINICAL CARE OR TO PARTICIPATE IN THE CLINICAL TRIAL. BUT THE DISPARITIES IN TERMS OF REFERRALS, AND THERE ARE A NUMBER OF PUBLICATIONS ON THIS, ARE PRETTY SUBSTANTIAL. AFRICAN-AMERICAN PATIENTS, PATIENTS WHO ARE ON MEDICAID, AND OTHER UNDERREPRESENTED POPULATIONS HAVE DIFFERENT REFERRAL RATES FOR TREATMENTS OF VARIOUS KINDS. IF YOU'RE INTERESTED IN THE PAPERS, I'VE SHARED SOME OF THEM WITH GEOFF.

IN FACT, I HAVE A CIRM-FUNDED UNDERGRADUATE IN THE TRAINING PROGRAM HERE THAT IS WORKING ON THIS, WHICH IS HOW WE DISCOVERED A NUMBER OF THESE PAPERS.

I'M NOT A CLINICIAN, I'M NOT AN EXPERT, BUT THE CONCERN I RAISED WAS FIGURING OUT A WAY TO ENSURE THAT THE CCCE'S WERE GOING TO HAVE, AND PERHAPS THE ALPHA CLINICS, WERE GOING TO PUT INTO

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THEIR PROPOSALS SPECIFIC WAYS IN WHICH THEY WERE GOING TO IMPACT REFERRAL. BECAUSE I THINK WITHOUT THAT, THE REFERRALS ARE LEFT TO THEIR OWN DEVICES, AND WE'LL BE IN THE SAME SITUATION THAT WE'RE IN NOW IN TERMS OF OUR HEALTHCARE SYSTEM. AND THAT'S NOT INCLUSION. SO THAT'S ONE THING.

AND SO I'M GLAD THAT, GEOFF, IN YOUR LANGUAGE YOU TALKED ABOUT A PURPOSEFUL COMPONENT TO THIS, WHICH I THINK IS INCLUDING THAT AS PART OF THE REQUIREMENT FOR APPLICATION. HOW ARE YOU GOING TO IMPACT REFERRALS? BECAUSE WITHOUT REFERRALS, NOTHING ELSE FOLLOWS. AND THERE'S A REFERRAL COMPONENT THAT SHOWS DISPARITIES, BUT THERE'S ALSO A COMPLETION OF A VISIT THAT SHOWS EVEN WORSE DISPARITIES. SO A PATIENT MAY BE REFERRED, BUT THEY MAY NOT GO.

SO THESE ARE ISSUES THAT I THINK ARE REALLY CENTRAL TO WHETHER THIS PROGRAM IS GOING TO SUCCEED OR NOT. AND I THINK THAT THE APPLICANTS NEED TO PUT THEIR HEADS TOGETHER AND FIGURE OUT HOW TO IMPACT THAT IN A WAY THAT NO ONE ELSE HAS DONE BEFORE.

THE OTHER THING THAT, WHICH I THINK YOU'VE INCLUDED, GEOFF, WHICH I THINK IS REALLY IMPORTANT, IS CIRM HAS TO MONITOR THIS, IN MY OPINION, A LOT

MORE CAREFULLY THAN YOU'RE MONITORING A DISCOVERY GRANT PROJECT OR SOMETHING LIKE THAT AND THEN HAVE THE ABILITY FOR THE CCCE'S TO ADAPT AND ADJUST. MESSAGE TESTING, DETERMINING WHETHER THE EDUCATION PART IS ACTUALLY IMPACTING OR HAVING -- THERE'S UPTAKE IN SPECIFIC COMMUNITIES. AS WE KNOW, THAT VARIES QUITE A BIT IN TERMS OF WHETHER MESSAGING WORKS OR NOT. I THINK THAT HAS TO BE A COMPONENT AS WELL RATHER THAN LEAVING IT FOR ANNUAL PROGRESS REPORTS WHERE THE NUMBERS LOOK SAD.

AND I'M NOT -- I DON'T KNOW WHAT THE TIME FRAME IS IN TERMS OF HOW OFTEN ONE WANTS TO LOOK AT THIS. BUT SOMEBODY WHO HAS PUBLIC HEALTH EXPERIENCE BROUGHT INTO CIRM TO LOOK AT THIS. BECAUSE THIS IS A HUGE HEAVY LIFT, NOT IN TERMS OF MONEY, BUT IN TERMS OF THE VISIBILITY OF THIS. I THINK HAVING SOMEBODY WHO HAS THAT EXPERTISE, WHOSE SOLE JOB IS TO MONITOR HOW THE INTERFACE IS OCCURRING BETWEEN THE CCCE'S AND THE ALPHA CLINICS, THE MESSAGING, THE PROGRESS THAT EACH OF THESE SITES ARE MAKING, THAT TO ME IS A REALLY IMPORTANT COMPONENT OF THIS AS WELL. I'LL STOP THERE.

CHAIRMAN GOLDSTEIN: GEOFF, I THINK PAT HAS RAISED SOME REALLY SERIOUS ISSUES. IS THERE SOME WAY TO LINK THE CALL FOR APPLICATIONS TO THE

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DISCUSSION FROM THIS GROUP BECAUSE I THINK THEY MAY FIND IT HELPFUL?

DR. LOMAX: YEAH. I CERTAINLY -- I THINK WE DO HAVE THE INTENTION OF LINKING -- A RESOURCE PAGE THAT WILL LINK TO A NUMBER OF THESE CONVERSATIONS. SO ABSOLUTELY BECAUSE THERE'S HIGH VALUE THERE.

I THINK THE REFERRAL, THE DISPARITY IN REFERRALS WILL, AGAIN, GIVEN THAT IS SOMETHING THAT REALLY COULD BE LAID OUT AS A KIND OF CENTRAL PROBLEM STATEMENT IN THE FRAMING OF THE APPLICATION. AND THEN ABSOLUTELY THE GWG WOULD BE LOOKING AT EVALUATING THE QUALITY OF THOSE PROPOSALS IN TERMS OF WHAT THEY PERCEIVE TO BE THE EFFICACY OF BEING ABLE TO ADDRESS THAT GAP.

ONE PARTICULAR PIECE THAT I DID FAIL TO MENTION IN TERMS OF FOLLOW-UP ON THAT ISSUE, TO WHAT EXTENT, AND I DON'T KNOW THE ANSWER TO THIS, THAT PRIMARY PHYSICIANS CAN PLAY A ROLE IN BRIDGING THAT GAP. THERE ARE ALREADY, AGAIN, WITHIN THE ALPHA CLINICS NETWORK SOME VERY TARGETED PROGRAMS THAT AIM TO MAKE CLINICAL RESEARCH VISIBLE TO PRIMARY CARE PHYSICIANS. LITERALLY THINGS LIKE POSTERS ON THE WALL AND MATERIALS LIKE THAT THAT HAVE GONE THROUGH EXTENSIVE FIELD TESTING. SO TRYING TO BRIDGE THESE

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RESOURCES INTO THIS PROGRAM, I THINK, ARE OPPORTUNITIES.

AND OUR CHALLENGE IS GOING TO BE MAKING THOSE CONNECTIONS OVER THE COURSE OF THE APPLICATION PERIOD AND CONNECTING KNOWN RESOURCES THAT HAVE THE POTENTIAL TO ADDRESS THE PROBLEMS WE'VE IDENTIFIED WITH APPLICANTS AND FACILITATE SOME OF THOSE PARTNERSHIPS.

CHAIRMAN GOLDSTEIN: TERRIFIC. CHRISTINE.

DR. MIASKOWSKI: THANKS SO MUCH. GEOFF, I REALLY APPRECIATE ALL THE HARD WORK THAT WENT INTO THIS PROPOSAL, AND I AGREE WITH SOME OF THE COMMENTS THAT PAT MADE.

I HAVE A COUPLE OF QUESTIONS. THE FIRST ONE, I JUST MAY HAVE MISSED IT. WHAT'S THE DEFINITION OF A COMMUNITY CARE CENTER? IS IT GEOGRAPHY? IS IT RELATIONSHIP OR DISTANCE TO AN ALPHA CLINIC? IS IT BEING CENTERED IN ETHICALLY DIVERSE COMMUNITIES? SO I'D LIKE TO HEAR A LITTLE BIT MORE ABOUT THAT, WHETHER THAT WAS CONSIDERED OR NOT.

AND THEN THE SECOND QUESTION MAYBE FITS WITH WHAT PAT WAS SAYING. YOU WERE SPEAKING ABOUT THE STAFF THAT WOULD NEED TO BE HIRED IN THESE SITES. I WAS WONDERING IF YOU CONSIDERED IN THE

CONCEPT PLAN SOME MINIMUM THRESHOLD FOR PATIENT REFERRALS THAT COULD BE LISTED IN THE APPLICATION THAT WOULD PROVE TO BE COST-EFFECTIVE. THERE WAS QUITE A LONG LIST OF PEOPLE THAT ARE NEEDED TO RUN THESE CENTERS AND/OR PROVIDE CARE. I THINK WE NEED TO HAVE SOME ESTIMATE OR EDUCATED GUESS MAYBE PERHAPS AT THIS POINT, OR YOU NEED TO WORK OUT WHAT WOULD BE THE MINIMUM THRESHOLD OF SERVICE THAT WOULD NEED TO BE PROVIDED TO MAKE THE BUDGET COST-EFFECTIVE.

I DON'T KNOW IF YOU HAD THOUGHT ABOUT THAT OR NOT.

AND THEN THE OTHER PERSON I WOULD LIST, BASED ON THE CONVERSATION WE HAD YESTERDAY, WHICH WAS WONDERFUL ACTUALLY, SOCIAL WORKERS ON THAT LIST OF PEOPLE WHO PROBABLY NEED TO BE HIRED.

DR. LOMAX: ON THE FIRST QUESTION, I THINK THERE WERE INITIALLY CONCERNS THAT -- WE DID GET QUESTIONS FROM SITES SAYING IS GEOGRAPHY GOING TO -- IS THERE SORT OF SOME KIND OF CALCULATION ON GEOGRAPHY GOING BE DETERMINANT OF WHO CAN APPLY. WE CURRENTLY DO NOT HAVE THAT. THE COUNTER ARGUMENT TO THAT OR THE COUNTER POINT TO JUST LOOKING AT A MAP AND SAYING HOW CLOSE OR HOW PROXIMATE OR DISTANCE THESE CENTERS ARE, THE EXAMPLE WAS GIVEN OF PARTS OF

SOUTH CENTRAL LOS ANGELES WHERE YOU HAVE INCREDIBLE POPULATION DENSITY THAT ARGUABLY, DESPITE THE PRESENCE OF MULTIPLE ALPHA CLINICS SITES, AT LEAST APPARENTLY ON A MAP, STILL REPRESENTS AN UNDERSERVED, UNDERRESOURCED COMMUNITY THAT IS NOT BEING REPRESENTED IN THESE TRIALS. AND THAT THERE ARE LOCATIONS THAT ARE UNIQUELY POSITIONED IN THAT SPACE TO CHANGE THAT.

SO GIVEN THAT DYNAMIC, WE DIDN'T -- WE REMAINED SILENT. IF YOUR RECOMMENDATION IS WE SHOULD DO OTHERWISE, THEN OBVIOUSLY WE WOULD ACT ON THAT RECOMMENDATION. BUT THOSE WERE THE TWO, I THINK, COMPETING BODIES OF EVIDENCE THAT WE WERE CONFRONTING DURING THE NEEDS ASSESSMENT THAT LED US TO REMAIN SILENT, IF THAT MAKES SENSE.

IN TERMS OF, I THINK, KIND OF THIS PERFORMANCE METRIC APPROACH AND MINIMUM STANDARDS, AGAIN, THOSE ARE CERTAINLY THINGS WE HAVE -- THEY ECHO HOW WE WOULD MILESTONE AND ADMINISTER PROGRAMS. BUT FROM THE STANDPOINT OF KNOWING KIND OF WHAT THAT METRIC IS, AT THE MOMENT I DON'T KNOW THE ANSWER TO THAT. BUT I APPRECIATE THE IDEA THAT WE NEED TO -- IF I UNDERSTAND THE POINT, WE SHOULD HAVE SOME REASONABLE ESTIMATE OF WHAT A REASONABLE PERFORMANCE STANDARD SHOULD BE AND THEN HOLD PEOPLE TO THAT

STANDARD. OTHERWISE WE'RE NOT ACHIEVING THE AIMS OF THE PROGRAM. AND I WOULD BE HAPPY TO ASK COLLEAGUES TO HELP DEVELOP THAT, OF GETTING THAT RIGHT.

AGAIN, I THINK FROM THE STANDPOINT OF LIKE SOCIAL WORKERS, THOSE GROUPS, AGAIN, SOCIAL WORKERS, COMMUNITY HEALTH WORKERS HAVE BEEN ENGAGED ALONG THIS WHOLE PROCESS, AND WE WILL CONTINUE TO ENGAGE THEM. I ENVISION THE STRUCTURE OF THIS IS REALLY DEVELOPED IN A WAY TO INCENTIVIZE APPLICANTS TO BRING THOSE SOCIAL WORKERS IN BECAUSE THE WEIGHT OF EVIDENCE TELLS US, ABSENT THEIR PARTICIPATION, WE'RE NOT GOING TO GET THERE. I THINK WE'RE PRETTY DEFINITIVE IN THAT POINT AT THIS STAGE.

I HOPE I COVERED, THAT'S RESPONSIVE.

DR. MIASKOWSKI: THANKS. I GUESS I WOULD LIKE TO SEE, EVEN IF WE DON'T HAVE AN ABSOLUTE METRIC, SOME ESTIMATION IN THE APPLICATION OF WHO THEY THINK THEY'RE GOING TO SERVE IN TERMS OF NUMBERS. BECAUSE I THINK FOR THE GRANTS WORKING GROUP IT'S GOING TO BE A CHALLENGE IF WE GET 15 APPLICATIONS AND WE DON'T HAVE A SENSE OF THE POTENTIAL IMPACT, I GUESS.

DR. LOMAX: I LOOK FORWARD TO WORKING WITH THE TEAM TO SORT OF -- I THINK THAT IS SOMETHING THAT'S VERY RIPE FOR BEING IN THE APPLICATION AND

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HOW TO GET THAT RIGHT. I WOULD CALL FOR HELP TO DO IT, BUT ALL IN ON MAKING IT HAPPEN.

DR. LEVITT: IF I CAN JUST FOLLOW UP JUST BRIEFLY ON THAT. SO WHEN YOU SUBMIT AN NIH GRANT, THERE ARE TABLES YOU HAVE TO PREPARE. AND THIS IS EXACTLY THE DATA THAT YOU HAVE TO PUT INTO THE GRANT. IT'S REQUIRED. WOMEN, UNDERREPRESENTED POPULATIONS, AND NUMBERS. YOU HAVE TO PUT IN NUMBERS LITERALLY, LIKE 27, 140, WHATEVER. AND YOU HAVE TO PUT IN THE GEOGRAPHIC COMPONENTS IN TERMS OF WHAT COMMUNITIES WHICH YOU ARE GOING TO RECRUIT AND HOW YOU'RE GOING TO RECRUIT. SO THOSE ARE ALREADY IN NIH GRANTS. AND SO YOU DON'T HAVE TO REINVENT THE WHEEL. YOU CAN LITERALLY USE THAT KIND OF APPROACH, MODIFY IT IF YOU WANT. BUT YOU CAN GET -- WHAT CHRISTINE SAID IS RIGHT. YOU CAN GET THOSE NUMBERS IN EVERY GRANT.

DR. MIASKOWSKI: JUST TO FOLLOW UP ON PAT. IN THE CURRENT GRANT APPLICATIONS ACROSS THE DIFFERENT CIRM INITIATIVES, THOSE TABLES EXIST FOR THE RECRUITMENT OF PATIENTS FOR THE TRIALS. SO I THINK THEY COULD BE MODIFIED TO ADJUST TO TYPE OF A SITUATION WHERE YOU'RE ANTICIPATING WHO THEY'RE GOING -- THE GEOGRAPHIC -- GEOGRAPHY AND ETHNIC DISTRIBUTION OF THE CATCHMENT AREA THEY PURPORT TO

SERVE. SO I DON'T THINK YOU HAVE TO REINVENT THE WHEEL, GEOFF. I THINK IF YOU TALK TO THE OTHER PEOPLE. GIL, FOR EXAMPLE, HE CAN HELP YOU WITH THAT.

CHAIRMAN GOLDSTEIN: GOOD. SO, GEOFF, I WOULD JUST ECHO WHAT PAT AND CHRISTINE HAVE BROUGHT UP. IT SEEMS LIKE AN ESSENTIAL ELEMENT OF ANY OF THESE PROPOSALS.

DEBORAH.

DR. DEAS: THANK YOU SO MUCH, GEOFF. IT'S REALLY A GREAT PRESENTATION. I'M VERY EXCITED ABOUT THE COMMUNITY CARE CENTERS OF EXCELLENCE.

AS WE STARTED ON THIS JOURNEY WITH THE LISTENING SESSIONS AND AREAS THAT ARE UNDERSERVED, SUCH AS THE INLAND EMPIRE AND THE CENTRAL VALLEY, WHAT I WAS THINKING, AND I THINK TO A LARGE EXTENT, WE WERE FOCUSING ON THOSE AREAS BECAUSE WE KNEW THAT THEY WERE UNDERREPRESENTED. AND WE WANTED MANY OF THOSE PATIENTS TO HAVE ACCESS TO SOME OF THE TREATMENTS AND TO THE CLINICAL TRIALS.

AS I THINK ABOUT THESE COMMUNITY CARE CENTERS OF EXCELLENCE, HOPEFULLY APPLICATIONS FROM THOSE AREAS, ONE OF THE CRITERIA, AND YOU'VE MENTIONED SOMETHING THAT MADE ME RECOGNIZE THAT YOU ARE SENSITIVE TO IT, THAT CRITERIA DEVELOPING THE

CAPACITY TO DELIVER INVESTIGATION OR REGULATORY MEDICATIONS DURING THE AWARD PERIOD.

DEPENDING ON WHERE THESE CENTERS ARE AND THE EXPERIENCE, I BELIEVE THAT THAT MAY BE DIFFICULT FOR SOME OF THESE AREAS BECAUSE THEY'RE JUST GETTING IN THE GAME, SO TO SPEAK. SO WE DEFINITELY WOULD NEED TO CONSIDER THAT AND NOT HAVE THAT AS A NEGATIVE IN THE APPLICATION PROCESS IF THEY DON'T APPEAR TO HAVE THAT POTENTIAL.

GOING BACK TO WHAT PAT STATED ABOUT THE REFERRAL, I BELIEVE TOO IT'S CERTAINLY KEY. AND PAT ELOQUENTLY HIGHLIGHTED THE DISPARITIES IN REFERRALS FOR UNDERREPRESENTED POPULATIONS, THE NEED TO, NOT ONLY EDUCATE THE COMMUNITIES, BUT ALSO WORK WITH CLINICIANS WHO ARE DOING THE REFERRALS. SO I REALLY BELIEVE THAT THERE SHOULD BE SOME DEMONSTRATION THAT ONE CAN DO THAT AS WELL AS AN INDICATION THAT THAT IS A PART OF WHAT THE CENTER IS PROPOSING TO DO AND HOW THEY MIGHT LOOK AT THE OUTCOME WITH WORKING WITH THESE CLINICIANS.

I ALSO BELIEVE THAT, WITH THE MENTION OF SOCIAL WORKERS, SOCIAL WORKERS ARE KEY. AND I ALSO BELIEVE THAT WHEN WE'RE WORKING, ESPECIALLY IN THIS AREA OF RESEARCH WHERE UNDERREPRESENTED POPULATIONS HAVE, RIGHTFULLY SO, SOME NEGATIVE PERCEPTIONS, THAT

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WE HAVE TO WORK HARD TO SORT OF MITIGATE THAT. AND IT MIGHT BE IMPORTANT TOO TO INCLUDE SOME BEHAVORIAL HEALTH CLINICIANS. WE CERTAINLY CAN HAVE THAT THROUGH SOCIAL WORKERS, BUT OTHER BEHAVORIAL HEALTH WORKERS TO WORK WITH PATIENTS WHO MAY BE POTENTIAL CANDIDATES FOR REFERRAL MIGHT BE APPROPRIATE AS WELL.

DR. LOMAX: THANKS FOR THAT. I BELIEVE IF WE, AGAIN, FRAME THIS AS WHAT'S EMERGING FROM THIS WORKING GROUP'S RECOMMENDATIONS IS THE REFERRAL GAP AS A PROBLEM STATEMENT IN THE APPLICATION THAT PRESUMABLY EXPERIENCE OR SITES THAT HAVE REALLY THOUGHT THIS THROUGH WOULD INCLUDE A BEHAVORIAL HEALTH COMPONENT. IT NEEDS TO GO PART AND PARCEL WITH HOW WE'RE TRYING TO MITIGATE THIS PROBLEM.

THERE'S BOTH A SOCIAL DETERMINANT ASPECT OF IT AS WELL AS TO SOME EXTENT THERE'S AN UNMET MEDICAL NEED. WE HAVE TRIED IN ALL ASPECTS TO PROVIDE FOR THAT FLEXIBILITY IN TERMS OF HOW WE WOULD THEN STRUCTURE -- I THINK THAT'S REFLECTED IN THE CONCEPT PLAN, AND WE'D CARRY THAT FORWARD TO THE APPLICATION.

CHAIRMAN GOLDSTEIN: INTERESTING. GEOFF, WHAT DO YOU IMAGINE ABOUT THE FREQUENCY OR NUMBER OF CALLS FOR APPLICATIONS YOU ARE GOING TO SET UP?

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CLEARLY YOU WILL HAVE A FIRST CALL. THERE WILL BE UNSUCCESSFUL APPLICANTS. WILL THERE BE A SECOND CALL A YEAR OR TWO LATER THAT ALLOW UNSUCCESSFUL APPLICANTS IN THE FIRST ROUND TO IMPROVE THEIR APPLICATIONS, DEAL WITH PROBLEMS SUCH AS WHAT WAS JUST DESCRIBED, AND COME IN FOR A SECOND CRACK AT THE APPLE?

DR. LOMAX: WELL, TO NOT GET -- I DON'T WANT TO GET OVER MY SKIS IN TERMS OF HAVING NOT -- I DON'T THINK WE'VE HAD THIS DISCUSSION WITHIN THE LEADERSHIP TEAM. BUT JUST LOOKING AT OUR PRECEDENT WITH THESE INFRASTRUCTURE PROGRAMS, AND WE'VE TENDED TO HAVE A MODEL THUS FAR WHERE THERE'S BEEN AN APPLICATION PHASE WITH THE OPPORTUNITY FOR IMPROVEMENT AND SORT OF A SECOND SHOT ON GOAL, IF YOU WILL. SO I'D PREFER TO DEFER TO THE OVERALL CIRM TEAM BEFORE SETTING EXPECTATIONS IN TERMS OF EXACTLY HOW WE WOULD MANAGE THAT APPLICATION PROCESS. BUT IF PRECEDENT IS A GUIDE, IT'S SIMILAR TO, I THINK, WHAT YOU JUST DESCRIBED, WHICH IS IT WOULDN'T BE ONE AND DONE, BUT SOME SORT OF PROCESS WHERE SITES COULD COME BACK AND ADDRESS ANY SORT OF WEAKNESS OR IMPROVE THOSE APPLICATIONS SO THAT THEY'RE DEEMED MERITORIOUS.

DR. THOMAS: GEOFF, COULD I JUST ADD. SO,

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LARRY, I WOULD ENVISION -- HELLO, EVERYBODY, BY THE WAY. I WOULD ENVISION SOMETHING SIMILAR TO WHAT WE DID WITH THE ALPHA CLINICS, WHICH WAS HAD THE FIRST ROUND, THEY GOT UP AND RUNNING, AND SUBSEQUENT TO THAT, IT WAS A REAPPLICATION PROCESS, WHICH IN THE ALPHA CLINICS EXPANDED THE SITES FROM FIVE TO NINE.

VICE CHAIR BONNEVILLE: THERE'S ALSO A BASIC SCIENCE BUDGET IN PROP 14 FOR THE COMMUNITY CARE CENTERS. AND THIS CONCEPT PROPOSAL DOES NOT ACTUALLY GO TO THE MAXIMUM OF WHAT THE BUDGET IS. SO THERE WILL BE MONEY TO APPLY TOWARDS CCCE'S IN THE FUTURE OR THEN HOWEVER WE WANT TO ADMINISTER THAT.

CHAIRMAN GOLDSTEIN: THIS ALL SEEMS PARTICULARLY IMPORTANT GIVEN THAT THIS IS A BRAND NEW PROGRAM THAT WE'VE NEVER DONE, AND WE DON'T KNOW WHAT THE APPLICATIONS ARE GOING TO LOOK LIKE AND HOW THEY'RE GOING TO GET REVIEWED. SO I'M GLAD TO HEAR THAT THERE'S AN OPPORTUNITY FOR SOME REFINEMENT AND IMPROVED PERFORMANCE.

DR. LOMAX: JUST TO ADD TO WHAT CO-CHAIR BONNEVILLE SAID, MY UNDERSTANDING, AND I'M SURE THE LAWYERS IN THE ROOM WILL CORRECT ME IF I'M SPEAKING OUT OF TURN, IS THAT THE ALLOCATION IN THE PROPOSITION REPRESENTS A FLOOR. AND THE BOARD DOES

HAVE THE DISCRETION TO RESOURCE THESE PROGRAMS AS THEY SEE NECESSARY. SO WE'RE BELOW THE FLOOR, SO TO SPEAK, AND THERE'S FLEXIBILITY MOVING FORWARD DEPENDING ON THE DETERMINATIONS OF THE BOARD.

CHAIRMAN GOLDSTEIN: I'LL JUST REMIND THE GROUP THAT WHEN THESE APPLICATIONS GET REVIEWED AND RECOMMENDED FOR APPROVAL OR NOT, THIS GROUP WILL SEE THEM BEFORE THEY GO TO THE BOARD FOR FINAL SIGN-OFF. SO THAT WILL GIVE US SOME SENSE OF WHAT SUCCESSFUL APPLICATIONS LOOK LIKE AND WHAT REVISIONS MIGHT LOOK LIKE IF WE WERE TO ORGANIZE A SECOND ROUND.

OTHER QUESTIONS OR DISCUSSION POINTS FOR GEOFF ON THIS TOPIC? ANYBODY ON THE PHONE? SO PUBLIC COMMENT?

MR. TOCHER: LARRY, WE JUST NEED A -- WAITING FOR A MOTION, I BELIEVE.

CHAIRMAN GOLDSTEIN: OKAY. COULD SOMEBODY MAKE A MOTION?

DR. GASSON: SO MOVED.

VICE CHAIR BONNEVILLE: SECOND.

CHAIRMAN GOLDSTEIN: OKAY. MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: JUST A NUMBER OF CLARIFICATIONS ON THE MOTION. I THINK THE UNDERLYING FUNDAMENTAL PROGRAM AND EFFORT IS

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OUTSTANDING. BUT WANTED TO CLARIFY THERE WERE A NUMBER OF VERY COGENT COMMENTS THAT WERE MADE THAT WOULD SEEM TO ANTICIPATE IMPROVING THIS TO THE NEXT LEVEL. SO JUST WANTED TO UNDERSTAND ARE THOSE COMMENTS EFFECTIVELY EMBEDDED IN A WAY THAT THEY'RE CLEAR AND THIS IS FUNCTIONALLY PART OF THE APPROVAL OF THE MOTION? SO JUST WANTED TO CORRELATE THE EXCELLENT REMARKS THAT HAVE BEEN MADE TO ENSURE THAT THOSE ARE INCORPORATED IN WHAT WE'RE APPROVING HERE.

MR. TOCHER: FROM A PROCESS STANDPOINT, THE MOTION CAN BE STATED AS TO RECOMMEND APPROVAL OF THE CONCEPT PLAN INCORPORATING CHANGES AND IMPROVEMENTS BASED ON THE DISCUSSION TODAY.

MR. FISCHER-COLBRIE: ARE THOSE IMPROVEMENTS SUFFICIENTLY DELINEATED THAT WE KNOW WHAT THOSE ARE, OR IS THERE ENOUGH SENSE FROM THE NOTETAKING FROM THE DISCUSSION THAT WE SHOULD FEEL CONFIDENT THAT THAT WILL GET CARRIED FORWARD, OR WHAT'S YOUR THOUGHT THERE?

MR. TOCHER: I THINK WHAT WE CAN DO IS WE CAN CONSIDER THE MOTION AS PHRASED, AND THEN WE WILL REVISE THE PROPOSAL TO INCORPORATE THOSE SUGGESTIONS, ENSURE THAT IT CAPTURES IT IN THE MEANTIME BEFORE IT COMES TO THE FULL BOARD ON THE 25TH.

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MR. FISCHER-COLBRIE: EXCELLENT. THANK YOU. THANKS FOR THE CLARIFICATION. APPRECIATE IT.

CHAIRMAN GOLDSTEIN: DEBORAH.

DR. DEAS: THAT ANSWERED MY QUESTION BECAUSE I JUST WANTED TO KNOW WHETHER THOSE SUGGESTIONS WOULD BE DELINEATED AND WE WOULD KNOW EXACTLY WHAT THEY WERE, BUT HE STATED THAT THEY WILL COME WHEN WE HAVE THE FULL BOARD MEETING FOR APPROVAL.

MR. TOCHER: CORRECT.

DR. DEAS: THANK YOU.

CHAIRMAN GOLDSTEIN: NOTHING FURTHER FROM THE COMMITTEE IN ATTENDANCE. SCOTT, MAY I NOW GO TO PUBLIC COMMENT?

MR. TOCHER: ABSOLUTELY. IT LOOKS LIKE WE HAVE ONE MEMBER WITH THEIR HAND RAISED, STEPHANIE FARRELL.

DR. FARRELL: HI, THIS IS STEPHANIE FARRELL FROM EISENHOWER. AND I SUPPORT CLINICAL TRIALS IN A COMMUNITY HOSPITAL SETTING. AND I'M WONDERING IF WE'VE LOOKED AT THE CLINICAL TRIALS THAT THE COMMUNITY CENTERS WOULD BE EXPECTED TO OPERATIONALIZE. PARTICULARLY WHEN YOU'RE DOING CLINICAL TRIALS WITH AN INVESTIGATIONAL DRUG, THERE'S A SITE QUALIFICATION PROCESS AND A SITE

EVALUATION QUESTIONNAIRE THAT'S FILLED OUT JUST TO MAKE SURE THAT YOU HAVE THE RESOURCES AND STAFF. AND I'M WONDERING IF WE'VE LOOKED AT THAT FOR THE COMMUNITY CENTERS.

WHEN THE IRB LOOKS AT THESE AT STARTING THE CLINICAL TRIAL AT YOUR SITE, THEY WANT TO KNOW THAT THE INVESTIGATOR, THE DOCTOR'S EXPERIENCED, AND THEY HAVE TRAINED STAFF. SO HAVE WE LOOKED AT THAT FOR THE COMMUNITY CENTERS?

DR. LOMAX: I'M HAPPY TO RESPOND TO THAT IF YOU LIKE, DR. GOLDSTEIN.

SO THE NUMBER OF SITES THAT, AGAIN, PARTICIPATED IN THE WORKSHOP AND THE NEEDS ASSESSMENT HAVE GONE THROUGH SITE QUALIFICATION PROCEDURES. THEY'VE, AGAIN, DEMONSTRATED CAPACITY TO SERVE CLINICAL TRIALS, NOT NECESSARILY IN CELL AND GENE THERAPY EXCLUSIVELY, BUT THEY HAVE THAT CAPACITY. SO THE SHORT ANSWER IS YES, AND WE THINK THAT THAT IS WHY THAT LANGUAGE IS ACTUALLY WRAPPED INTO THE ELIGIBILITY CRITERIA, IS THERE SUCH NUMBER OF SITES THAT HAVE THAT CAPACITY AT A LEVEL WHICH WOULD BE SATISFACTORY, I THINK, TO CIRM IN TERMS OF EXPECTATIONS AND QUALITY. AND SO THERE ARE SITES THAT HAVE DONE THOSE PROCESSES.

AGAIN, I CITE ONCOLOGY WHICH IS AN AREA

WHERE I THINK THERE'S THE MOST EXPERIENCE. AND I THINK ONCOLOGY IS IMPORTANT BECAUSE IT DOES TOUCH ON A LOT OF THE WORK GOING ON IN CELL AND GENE THERAPY SPACE. SO IT'S A GOOD INDICATOR OF SITE READINESS.

DR. FARRELL: THANK YOU.

CHAIRMAN GOLDSTEIN: OTHER PUBLIC COMMENT ON THE LINE?

MS. MANDAC: THERE ARE NO OTHER HANDS RAISED.

CHAIRMAN GOLDSTEIN: ANY FINAL SUGGESTIONS OR QUESTIONS FROM THE SUBCOMMITTEE? IF NOT, SCOTT, MAY I CALL THE QUESTION AND GET US TO VOTE?

MR. TOCHER: ABSOLUTELY. MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: MONICA CARSON.

DR. DEAS: SHE LEFT. YES.

MR. TOCHER: SORRY, DEBORAH.

MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YES.

MR. TOCHER: JUDY GASSON.

DR. GASSON: YES.

MR. TOCHER: LARRY GOLDSTEIN.

CHAIRMAN GOLDSTEIN: YES.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: PAT LEVITT.
DR. LEVITT: YES.
MR. TOCHER: CHRISTINE MIASKOWSKI.
DR. MIASKOWSKI: YES.
MR. TOCHER: KAROL WATSON.
DR. WATSON: YES.
MR. TOCHER: KEITH YAMAMOTO.
DR. YAMAMOTO: YES.
MR. TOCHER: THANK YOU VERY MUCH. THE MOTION CARRIES.
CHAIRMAN GOLDSTEIN: CAN I JUST TAKE A MOMENT TO CONGRATULATE GEOFF, MARIA, AND ANYBODY ELSE WHO WORKED ON THIS. THIS IS A NEW AREA OF ENDEAVOR. I THINK YOU GUYS HAVE DONE A GOOD JOB OF SETTING IT UP. OF COURSE, WE DON'T REALLY KNOW WHAT'S GOING TO COME IN, BUT YOU'VE POSITIONED THIS, I THINK, FOR SUCCESS. AND SO CONGRATULATIONS.
DR. LOMAX: THANKS SO MUCH. I'D LIKE TO JUST GIVE A QUICK SHOUT-OUT TO EMILY REYES. WITH THE NEEDS ASSESSMENT, SHE WAS A REAL MOVER AND SHAKER IN TERMS OF MAKING THAT HAPPEN. SO THANK YOU.

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CHAIRMAN GOLDSTEIN: SO STATED. OKAY. I NOW RECUSE MYSELF AND HAND THE FLOOR OVER TO SCOTT.

MR. TOCHER: GREAT. THANKS, LARRY. BEFORE I HAND THIS TO MARK WHO WILL LEAD THE SUBSTANTIVE DISCUSSION, I JUST WANT TO PROVIDE A COMMENT ON THE BOUNDARIES AND EXPECTATIONS FOR THIS NEXT AGENDA ITEM.

AS YOU CAN SEE FROM THE BRIEFING MEMO, THIS IS A POLICY DISCUSSION THAT AROSE BASED ON THE APPLICATION REVIEW SUBCOMMITTEE'S CONSIDERATION OF AN APPLICATION AT ITS LAST MEETING IN NOVEMBER. AND THE APPLICATION REVIEW SUBCOMMITTEE HAS PAUSED ITS CONSIDERATION OF THAT APPLICATION PENDING THE POLICY DISCUSSION TO OCCUR TODAY AND THEN BASED ON THIS DISCUSSION, AT THE FULL BOARD MEETING ON JANUARY 25. AT THAT TIME THE APPLICATION REVIEW SUBCOMMITTEE, WHICH IS CHARGED WITH REVIEWING RECOMMENDATIONS FROM THE GRANTS WORKING GROUP AND MAKING FUNDING DECISIONS, WILL RESUME ITS DELIBERATIONS ON THAT PARTICULAR AWARD.

SO THE PURPOSE OF THIS MEETING AND THIS DISCUSSION IS NOT TO DETERMINE THE MERITS OF THE APPLICATION THAT GIVES RISE TO THE DISCUSSION TODAY, THAT WILL BE THE PURVIEW OF THE ARS, BUT RATHER TO ENGAGE IN THE POLICY DISCUSSION AND GUIDANCE ON THE

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SUBJECT. SO WITH THAT, I HOPE THAT'S HELPFUL. TURN IT OVER TO MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: THANK YOU, SCOTT. AND, AGAIN, JUST TO REITERATE, WHEREAS THIS CAME INITIALLY FROM AN INITIAL PROPOSAL, WE'RE VERY MUCH INTERESTED IN TRYING TO DISCUSS AND REVIEW WHAT SHOULD BE UNDER CONSIDERATION FOR A MORE FORMAL POLICY BY CIRM. AND WITHIN THAT CONTEXT, THAT HAS TO DO WITH THE RUBRIC, IF YOU WILL, OF N OF 1 TRIALS, WHICH IS A LITTLE BIT OF A MISNOMER, BUT IS IMPORTANT FOR CONSIDERATION IN THE CONTEXT THAT, WITH VARIOUS DEFECTS, IF YOU WILL, THERE'S THE OPPORTUNITY FOR MATERIALS OF ANTISENSE OLIGONUCLEOTIDES TO BE ABLE TO OBVIATE THE UNDERLYING PROBLEMS CAUSE OF DISEASE FOR A PARTICULAR INDIVIDUAL.

WITHIN THAT CONTEXT, WE'RE GENERALLY TALKING EXTREMELY RARE CONDITIONS IN THE CONTEXT THAT ESSENTIALLY THIS BECOMES NEARLY PATIENT SPECIFIC WITH ONE OR A VERY SMALL HANDFUL OF PATIENTS THAT THAT PARTICULAR APPROACH FOR A THERAPY MIGHT BE APPLIED.

AND SO JUST TO FRAME THAT A LITTLE BIT, AND THIS IS A SITUATION FROM THE MATERIALS PROVIDED, YOU CAN SEE ONE NON-PROFIT ORGANIZATION THAT HAS,

FOR EXAMPLE, EVALUATED 240 PATIENTS. THEY'VE DONE A PARTICULAR SCREENING AROUND GENOTYPE AND PHENOTYPE THAT HAS LED TO THE PROSPECT OF A HUNDRED PATIENTS THAT MIGHT BENEFIT FROM DEVELOPMENT OF THE ANTISENSE OLIGONUCLEOTIDES OR ASO'S. AND THAT'S IN THE CONTEXT THAT THE CURRENT COST FOR GIVING THAT IS $1.5 MILLION. AND WHEREAS, THAT IS RELATIVELY INEXPENSIVE COMPARED TO A DRUG DEVELOPMENT PROGRAM. IT'S ALSO A CONDITION WHERE OBVIOUSLY THE NUMBER OF PEOPLE THAT CAN BENEFIT FROM THAT ARE LIMITED.

AND YOU CAN SEE THE SITUATION OF, WELL, IF THERE'S AN INITIAL WAVE OF A HUNDRED PATIENTS AT 1.5 MILLION EACH AND THERE COULD BE MANY, MANY MORE WAVES OF THAT, THE OVERALL COST WOULD AGGREGATE TO ONE THAT WOULD SWAMP THE OVERALL ALLOCATION OF FUNDING FROM CIRM.

SO IT LED TO THE DISCUSSION, THEN, ABOUT WHAT ARE CONSIDERATIONS THAT CIRM MIGHT WANT TO TAKE IN MIND RELATED TO PROPOSALS THAT COME IN AROUND ASO'S AND HOW CIRM MIGHT SUPPORT THOSE OR NOT DEPENDING ON THE UNDERLYING CONDITIONS. THE MATERIALS PROVIDED, THERE ARE UNDERLYING BENEFITS LISTED IN TERMS OF THE ABILITY TO HAVE A BETTER UNDERSTANDING OF MECHANISMS OF ACTION OF DISEASE THAT, IN TURN, COULD HAVE A MORE FAVORABLE OUTCOME

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FOR ASSESSMENT OF OTHER DISEASE CONDITIONS, THE ABILITY TO HAVE SELECTION OF PATIENTS THAT THERE MIGHT BE TRUE CLINICAL BENEFITS.

IN OTHER WORDS, IN SOME CASES THIS PARTICULAR THERAPY, IF YOU WILL, DOESN'T ACTUALLY -- WHEREAS, IT WILL CURE THE UNDERLYING PROCESS OR MITIGATE THE UNDERLYING PROCESS OF DISEASE, IN SOME CASES THE PATIENTS WON'T GET A TREMENDOUS CLINICAL BENEFIT BECAUSE THEY ALREADY HAVE THE CONSEQUENCES OF THE UNDERLYING CONDITION OVERALL. BUT OBVIOUSLY THERE'S AN ATTEMPT TO HAVE SELECTION OF PATIENTS FOR WHICH THERE IS THE OPPORTUNITY FOR STRONG CLINICAL EFFECT.

THERE ARE OTHER ADVANTAGES LISTED. ONE GROUP INDICATES THAT THEY HAVE A CLIN2 APPROACH FROM CIRM IN TERMS OF TESTING PRECLINICAL EFFICACY OF AN ASO TO HELP POTENTIALLY EVEN SPEED THE PROCESS FURTHER FROM 18 MONTHS AS WELL AS REDUCE THE COST TO 700,000, WHICH IS THEIR TARGET. SO THERE ARE DEFINITE ADVANTAGES AROUND THIS, AND THEN THERE ARE CONCERNS. AND THERE IS RECOGNITION THAT WE DON'T REALLY HAVE A GOOD HANDLE ON HOW TO THINK ABOUT THESE THINGS, HOW WE MIGHT EVALUATE THEM, HOW DOES THAT FIT INTO THE OVERALL SCHEMA FOR FUNDING ALLOCATION AND HENCE PROVIDE THIS INFORMATION TO THE

SCIENCE SUBCOMMITTEE AND BE ABLE TO HAVE A DISCUSSION AROUND ISSUES AND OPPORTUNITIES ASSOCIATED WITH THIS THAT THEN, IN TURN, MIGHT BE FORMULATED INTO A MORE STRUCTURED POLICY DETERMINATION OR NOT DEPENDING ON WHAT THE OUTCOME IS.

SO THIS IS A LITTLE BIT OF BACKGROUND ON KIND OF WHAT'S ON THE TABLE. ANYBODY ELSE LIKE TO AMPLIFY THAT OR CLARIFY MY COMMENTS BEFORE WE GET INTO THE DISCUSSION IF THERE ARE ANY OTHER COMMENTS TO THAT SET OF CIRCUMSTANCES. OKAY. NOT HEARING ANY, I THINK -- SO RIGHT NOW WE'D LIKE TO THROW IT OUT TO THE SCIENCE SUBCOMMITTEE AND GET FEEDBACK AND DISCUSSION AROUND CONSIDERATIONS AROUND WHAT MIGHT BE DONE FROM THE POLICY PERSPECTIVE OR, AGAIN, NOT JUST EVALUATE EVERY PROPOSAL AS IT COMES IN THROUGH THE NORMAL REVIEW PROCESS. SO IF PEOPLE WOULD LIKE TO COMMENT ON THE COMMITTEE, THAT WOULD BE GREAT.

DR. GLEESON: IF YOU'D LIKE, MY NAME IS JOSEPH GLEESON. I'M A PROFESSOR AT THE UNIVERSITY OF CALIFORNIA HERE TO ANSWER QUESTIONS. I'D BE HAPPY TO PROVIDE ANY MORE CONTEXT, BUT DON'T WANT TO INTERRUPT ANY OF YOUR NORMAL PROCEEDINGS.

MS. MANDAC: SORRY, DR. GLEESON. WE'RE NOT QUITE READY FOR PUBLIC COMMENT YET.

MR. FISCHER-COLBRIE: DR. GLEESON IS ONLINE. I THINK ALSO I SAW EARLIER ANOTHER DOCTOR, PROBABLY NOT GOING TO PRONOUNCE THE NAMES RIGHT, BUT MIGNON IS ALSO ON THE LINE. I SEE YOU'VE ALSO PROVIDED SOME INFORMATION. SO I SUSPECT WE CAN ASK THEM, BUT MARIA.

VICE CHAIR BONNEVILLE: I THINK THE ISSUE AT HAND IS ARE WE READY TO MOVE FORWARD FUNDING THESE SORTS OF PROGRAMS THAT COME IN WITHOUT AN OVERALL STRATEGY HOW WE'RE GOING TO DEAL WITH RARE DISEASE AND N OF 1 SPECIFICALLY. AND WITHOUT MORE INFORMATION FROM OUR INTERNAL TEAM AND ESPECIALLY AS WE MOVE FORWARD WITH PRIORITIZATION AS A WHOLE, I THINK IT'S DIFFICULT FOR US TO MAKE A FUNDING DECISION WITHOUT MORE CLARITY AS TO THE OVERALL GOALS OF CIRM LEADERSHIP. AND WAITING FOR THEM TO BRING A STRATEGY TO US, I THINK, IS THE BEST COURSE MOVING FORWARD SO THAT WE CAN UNDERSTAND HOW THIS ALL FITS TOGETHER.

SO I WOULD SAY THAT THAT'S MY VIEWPOINT. IT'S NOT THAT I DON'T BELIEVE THAT THESE AREN'T VALUABLE PROGRAMS THAT ARE COMING FORWARD, AND OBVIOUSLY THEY HELP, SO ULTIMATELY THAT IS WHAT WE'RE ALL STRIVING FOR. I JUST THINK WE NEED TO PUT A PAUSE MOMENTARILY IN ORDER TO HAVE THE TEAM BRING

FORWARD A STRATEGY THAT WE CAN ADOPT.

MR. FISCHER-COLBRIE: THANKS, MARIA. J.T., I SEE YOUR HAND UP.

DR. THOMAS: FURTHER TO WHAT MARIA JUST SAID, I'VE ALREADY IDENTIFIED THE BROADER ISSUE OF HOW WE'RE GOING TO BE APPROACHING RARE DISEASE GOING FORWARD AS A MATTER TO BE TAKEN UP BY THE LEADERSHIP TEAM IN A VERY NEAR-TERM MEETING. SO PRECISELY WHAT SHE'S TALKING ABOUT IS SOMETHING WE'RE GOING TO BE FOCUSING ON AND PREPARED TO BRING BACK TO THIS SUBCOMMITTEE THE UPSHOT OF THOSE DISCUSSIONS.

MR. FISCHER-COLBRIE: THANKS, J.T. I THINK THAT'S EXTREMELY IMPORTANT CONTEXT BECAUSE IT CALLS INTO QUESTION THE VERY NARROW CONSIDERATION OF N OF 1 AND LEADS RIGHT TO THE QUESTION OF WHAT ABOUT AN N OF 10? WHAT IF -- WHERE IS THE CUTOFF, IF YOU WILL, IF THERE ARE POLICY DECISIONS, N OF 50 OR HOW THAT MIGHT COME INTO PLAY IN WHAT ARE CONSIDERATIONS AROUND FOCUS AREAS THAT SHOULD HAVE FURTHER EMPHASIS. SO THANK YOU FOR THOSE CLARIFYING COMMENTS TO GIVE THE OVERALL LANDSCAPE BY OPENING UP THE APERTURE FOR THE VERY BROAD DISCUSSION AROUND FOCUS AREAS.

SO, CHRISTINE, COMMENTS, QUESTIONS?

DR. MIASKOWSKI: THANK YOU. I WOULD LIKE

TO SUPPORT MARIA AND J.T.'S SUGGESTION. I READ THE MATERIALS THAT WERE PROVIDED. AND IN MY OWN MIND, I WAS GOING THROUGH KIND OF THE RISK/BENEFITS TO THE INDIVIDUAL WHO'S SUFFERING WITH THIS AND THEIR FAMILY MEMBER AND THEN OUR ORGANIZATION. AND I REALLY BELIEVE WE NEED A CAREFUL LOOK AT THIS IN TERMS OF THE WHOLE PICTURE.

I'D BE INTERESTED TOO WHAT OUR PREVIOUS EXPERIENCE HAS BEEN FUNDING THESE N OF 1 TRIALS AND WHAT'S BEEN THEIR SUCCESS OR NOT. IT TAKES A LITTLE MORE STUDY FROM MY PERSPECTIVE.

MR. FISCHER-COLBRIE: GREAT. PAT.

DR. LEVITT: I DON'T HAVE TO REPEAT WHAT CHRISTINE JUST SAID BECAUSE I WAS GOING TO SAY THE SAME THING, THAT THERE'S JUST COMPONENTS OF THIS THAT ARE REALLY COMPLICATED FROM MY PERSPECTIVE. AND I NEED MORE INFORMATION TO SORT OF THINK THROUGH THIS. THERE ARE ETHICAL, PRACTICAL CONSIDERATIONS. THERE ARE CONSIDERATIONS AROUND PRIORITIES FOR THE ORGANIZATION. AND INTEGRATING THOSE RIGHT NOW IN MY BRAIN IS NOT HAPPENING.

SO THIS MEETING THAT J.T. REFERRED TO, I THINK, IS REALLY IMPORTANT IN TERMS OF GETTING SOME OF THIS INFORMATION TO THE SUBCOMMITTEE TO MAKE SOME SORT OF RECOMMENDATION.

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MR. FISCHER-COLBRIE: OKAY. OTHER COMMENTS BY MEMBERS OR ANYBODY ON THE CALL OTHER THAN THE PUBLIC RIGHT NOW BECAUSE WE NEED TO FORMALLY OPEN THAT SUBSEQUENTLY. SO KEY MEMBERS OR STAFF, ANY ADDITIONAL COMMENTS?

SO TO SUMMARIZE, MY SENSE IS THAT THERE'S NO PARTICULAR DECISION OR DETERMINATION TODAY WITH RESPECT TO POLICY, THAT THERE'S UPCOMING STAFF AND OTHER MEETINGS THAT IS EXPECTED TO HELP LEAD TO A BROADER DISCUSSION AROUND FOCUS AREAS AND HOW DIFFERENT ELEMENTS OF A VARIETY OF TRIALS CAN FIT INTO THE OVERALL SCHEMA FOR WHAT NEEDS TO GET ACCOMPLISHED.

SO WITH THAT, MY ASSUMPTION IS THERE'S NO PARTICULAR MOTION ON THE TABLE CURRENTLY FOR REVIEW; BUT IN ANY CASE, WE WANT TO ALLOW THE PUBLIC TO MAKE COMMENTS ALONG THE WAY. SO I THINK THE NEXT STEP WOULD BE TO ALLOW PUBLIC COMMENTS.

MR. TOCHER: WE HAVE A COMMENT FROM MARIA BONNEVILLE FIRST.

VICE CHAIR BONNEVILLE: MARK, I THINK PART OF TALKING TODAY WAS BEING ABLE TO GUIDE THE ARS MOVING FORWARD AS TO HOW WE WOULD APPROACH APPLICATIONS LIKE THIS THAT COME TO THE GWG AND/OR WHETHER OR NOT THEY WOULD -- WE WOULD EVEN BRING

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THEM UP AS A POLICY MATTER, NOT THE POLICY, BUT WHAT A RECOMMENDATION WOULD BE TO THE APPLICATION REVIEW SUBCOMMITTEE MOVING FORWARD IF THESE APPLICATIONS WERE TO COME TO THEM. ARE WE WAITING FOR A BIGGER STRATEGY AND A BIGGER POLICY TO COME FORWARD FROM THE TEAM IN ORDER TO THEN MOVE FORWARD WITH FUNDING DECISIONS OR NOT? I THINK THAT'S THE GUIDANCE THAT WE SHOULD PROVIDE FOR THE APPLICATION REVIEW SUBCOMMITTEE.

MR. FISCHER-COLBRIE: YEAH, MARIA. THANK YOU FOR THE CLARIFICATION. SO INHERENTLY THAT IS THEN THE PHENOMENON OF BEING ABLE TO THINK ABOUT WHAT WE'RE GOING TO DO WITH PROPOSALS AS THEY COME THROUGH THE PROCESS CURRENTLY. BEFORE WE COMMENT ON THAT, GIL, I SEE YOU'VE GOT YOUR HAND UP.

DR. SAMBRANO: YEAH. IT WAS JUST AN EXPANSION ON THAT COMMENT, THAT I THINK IT WOULD BE GREAT TO HAVE SOME GUIDANCE IN TERMS OF WHAT WE DO AT CIRM IN TERMS OF ACCEPTING THESE APPLICATIONS. THERE'S A LOT OF WORK THAT GOES INTO THESE ON THE PART OF THE APPLICANT TO PUT AN APPLICATION TOGETHER. AND IF ULTIMATELY THE ARS OR THE BOARD IS NOT GOING TO BE ABLE TO CONSIDER IT, WE WOULD RATHER LET THEM KNOW AHEAD OF TIME BEFORE COMING IN.

SO IF THERE'S A DECISION OR SOME GUIDANCE

THAT COULD BE PROVIDED IN TERMS OF WHETHER WE ACCEPT SUCH APPLICATIONS FOR THE TIME BEING UNTIL WE DEVELOP A PROPOSAL OF EXACTLY WHAT TO DO, THAT MAY BE HELPFUL. THANK YOU.

MR. FISCHER-COLBRIE: OKAY. WE WILL THROW THAT OUT TO THE COMMITTEE FOR DISCUSSION ABOUT WHAT MIGHT BE DONE WITH THE THINGS EITHER -- NOT IN THE HOPPER, BUT GENERICALLY THINGS THAT COME DOWN THE PIPE HERE IN THE INTERIM WHILE THERE ARE CONSIDERATIONS BEING MADE FOR OVERALL FOCUS AREAS AND DISCUSSIONS. SO IF THE SCIENCE SUBCOMMITTEE WOULD LIKE TO COMMENT ABOUT THAT. PAT.

DR. LEVITT: YEAH. I THINK IF THERE'S A CONSENSUS FOR ASKING FOR INFORMATION FROM A NUMBER OF DOMAINS THAT WILL HAVE AN IMPACT ON THE RECOMMENDATION THAT'S GOING TO BE MADE, THEN I WOULD RECOMMEND WE MAINTAIN STATUS QUO. OTHERWISE, WE'RE GOING TO BE MAKING A CHANGE WITHOUT THE INFORMATION THAT A NUMBER OF US FEEL IS IMPORTANT. SO I DON'T SEE ANY REASON TO MAKE A CHANGE NOW. I'M NOT COMFORTABLE WITH MAKING A CHANGE WITHOUT THAT ADDITIONAL INFORMATION AND CONVERSATION THAT GOES ON WITH THE TEAM AND THE MEETING THAT'S GOING TO OCCUR.

SO I WOULD RECOMMEND THAT WE DON'T CHANGE ANYTHING RIGHT NOW. SO IF THAT MEANS WE'RE

ACCEPTING APPLICATIONS, WE'RE ACCEPTING APPLICATIONS, FROM MY PERSPECTIVE. I DON'T KNOW WHAT ELSE TO DO BECAUSE THEN YOU'RE ASKING US TO MAKE A POLICY DECISION WITHOUT THE INFORMATION.

MR. FISCHER-COLBRIE: THERE'S ALSO THE SCENARIO OF SAYING, HEY, SOME OF THESE APPLICATIONS CAN FUNCTIONALLY BE DELAYED, NOT DELAYED, BUT COULD BE ESSENTIALLY DELAYED WHILE THERE'S THE OVERALL DISCUSSION. BUT THAT'S A WELL-TAKEN POINT WITH RESPECT TO, IN THE ABSENCE OF A POLICY CONSIDERATION, THEN BUSINESS AS USUAL CAN MAKE SENSE.

I THINK I SAW CLAUDETTE.

MS. MANDAC: IT'S OKAY. THERE'S A HAND RAISED IN THE ROOM, BUT IT'S BEEN SAID. CHRISTINE HAS HER HAND RAISED THOUGH.

DR. MIASKOWSKI: I AGREE WITH PAT. I THINK WE SHOULD CONTINUE THE WAY WE ARE UNTIL THERE IS A FORMAL DISCUSSION. PEOPLE HAVE PUT WORK INTO THESE APPLICATIONS, AND WE SHOULD CONSIDER THEM UNTIL WE MAKE A FORMAL ANNOUNCEMENT REGARDING OUR POLICY.

MR. FISCHER-COLBRIE: MARIA.

VICE CHAIR BONNEVILLE: MY PRIMARY CONCERN IS THAT WE WOULD MOVE FORWARD AND PERHAPS FUND N OF

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1 APPLICATIONS AND THEN SET A PRECEDENT FOR HAVING DONE SO AND THEN GO TO A RARE DISEASE STRATEGY THAT PERHAPS APPROACHES THINGS DIFFERENTLY. THAT'S THE CONCERN.

IF THE GROUP DECIDES THAT THAT'S OKAY, I ACCEPT THAT DECISION. FROM MY PERSPECTIVE, WITHOUT HAVING A CLEAR UNDERSTANDING OF WHAT OUR STRATEGY IS SPECIFICALLY FOR N OF 1 APPLICATIONS, I DON'T KNOW HOW TO MOVE FORWARD WHEN THESE APPLICATIONS COME FORWARD AND ARE RECOMMENDED.

DR. LEVITT: CAN SOMEBODY, MAYBE GIL, SO WHAT'S IN THE HOPPER IN TERMS OF APPLICATIONS THAT HAVE COME IN? I'M NOT ASKING FOR SPECIFICS OF THE APPLICATIONS, BUT LIKE ARE THERE TEN, ARE THERE TWO? AND WHEN WAS THE ANNOUNCEMENT POSTED FOR ACCEPTING THESE APPLICATIONS?

DR. SAMBRANO: RIGHT. SO WE HAVE CURRENTLY ONE IN PROCESS. WE MAY GET MORE, BUT WE DON'T KNOW. WE'VE SEEN AN UPTICK IN APPLICATIONS. SPECIFICALLY N OF 1, THERE'S ONLY ONE THAT'S UNDER CONSIDERATION AT THE MOMENT. AND THERE'S NO SPECIFIC -- WE DIDN'T MAKE ANY SPECIFIC ANNOUNCEMENT TO ACCEPT OR NOT. THEY JUST FIT WITHIN WHAT OUR CURRENT ELIGIBILITY CRITERIA ARE.

DR. LEVITT: OKAY. ALL RIGHT.

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SO, MARIA, YOU'RE ASKING FOR -- ESSENTIALLY WHAT YOU'RE SAYING -- I DON'T SEE HOW WE CAN CONSIDER THE ONE THAT'S ALREADY THERE THAT'S IN THE HOPPER THAT IS GOING TO UNDERGO SCIENTIFIC REVIEW. BUT THEN YOU'RE ASKING US TO CONSIDER PAUSING ACCEPTING THEM? THAT WOULD TAKE AN ANNOUNCEMENT TO THE COMMUNITY, TO THE SCIENTIFIC COMMUNITY TO PAUSE. RIGHT?

VICE CHAIR BONNEVILLE: MY UNDERSTANDING FROM THE INTERNAL TEAM, AND PERHAPS ABLA CAN SPEAK TO THIS, IS THAT THERE ARE PLANS FOR MORE OF THESE TO COME IN. SHE'S HEARD FROM THE COMMUNITY. SO MY CONCERN WOULD THEN BE THAT WE HAVE AN INFLUX OF APPLICATIONS, WHICH, AGAIN, I'M NOT SUGGESTING NOBODY -- THAT THESE AREN'T VALUABLE, IMPORTANT.

DR. LEVITT: I UNDERSTAND.

VICE CHAIR BONNEVILLE: SO IT'S JUST ALL OF A SUDDEN WE GET TEN IN A MONTH, WHAT DO WE DO? AND SO I GUESS I'M UNDERSTANDING FROM THE INTERNAL TEAM, BECAUSE I HAVE HEARD CONCERNS FROM THEM ABOUT THIS, I THINK WOULD BE HELPFUL.

DR. CREASEY: THANK YOU, MARIA B. SO AS FAR AS THE CLINICAL DEVELOPMENT IS CONCERNED, THERAPEUTICS DEVELOPMENT, WE HAVE HAD A NUMBER OF INQUIRIES ABOUT N OF 1, WHETHER IT'S FOR ASO'S OR

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OTHERWISE. SO FOR THAT PURPOSE, REMEMBER WE CONDUCTED A RARE DISEASE WORKSHOP ON NOVEMBER 15TH IN WHICH WE BROUGHT IN EXPERTS FROM DIFFERENT AREAS. AND WE ARE ASSESSING THE RECOMMENDATIONS FROM ALL THE OTHER EXPERTS THAT ARE WORKING IN THE AREA. AND I HAVE RECOMMENDED TO THE LT THAT WE WAIT UNTIL WE ACTUALLY HAVE PUT TOGETHER THE STRATEGY FOR RARE DISEASES. WE'RE WORKING ON IT. IT'S NOT READY, BUT IT'S IMPORTANT THAT, IF WE'RE GOING TO CONSIDER AN N OF 1, WE NEED TO MAKE SURE THAT IT'S NOT RESTRICTED TO ASO'S AND THERE ARE OTHER POTENTIAL TECHNOLOGIES THAT CAN BE USED.

SO FOR THAT PURPOSE, IT'S PREMATURE TO SAY WE'RE JUST GOING TO FUND N OF 1 WITHOUT KNOWING REALLY HOW WE'RE GOING TO ALLOCATE OUR RESOURCES FOR THAT EFFORT. I JUST WOULD LIKE THE ATTENDEES TO BE AWARE OF THE FACT THAT WE ARE DILIGENTLY WORKING ON IT. WE HAVE A NUMBER OF RECOMMENDATIONS. THERE ARE VERY EXCITING AND INTERESTING TECHNOLOGIES OTHER THAN ASO'S. WE JUST, AGAIN, NEED TO FIGURE OUT DO WE SEGMENT THE RARE DISEASE POPULATION. HOW DO WE THEN FUND 100 VERSUS N OF 10 VERSUS N OF 1? ALL THAT IS PART OF OUR CURRENT THINKING.

I DON'T WANT TO SAY THAT WE ALREADY HAVE A STRATEGY. WE'RE WORKING ON IT. AND ONCE WE HAVE A

DRAFT, WE WILL SHARE IT WITH THE SCIENCE SUBCOMMITTEE. MOST IMPORTANT IS FOR PEOPLE TO RECOGNIZE THE FACT THAT WE APPRECIATE THEIR COMPASSION IN THE AREA OF N OF 1. GIVEN, THOUGH, OUR MANDATE AND BUDGET AND ALL OF THAT, WE HAVE TO MAKE SURE THAT WE ALLOCATE OUR RESOURCES IN A MANNER THAT ARE COMMENSURATE WITH OUR UNDERSTANDING OF ALL CAPABILITIES THAT COME TO AN N OF 1 OR OTHERWISE IN THE AREA OF RARE DISEASE.

SO I'M A PROPONENT OF THE FACT THAT WE WAIT TILL WE HAVE A STRATEGY. AND IT MAY BE -- MY RECOMMENDATION WOULD BE THAT WE ACTUALLY PUT A HOLD ON ACCEPTING N OF 1S TILL WE'VE HAD ACTUAL DISCUSSION OF THE STRATEGIC PLAN FOR RARE DISEASE. AND THEN IT MAKES SENSE THAT WE REMOVE THE HOLD.

VICE CHAIR BONNEVILLE: PAT, WE COULD CONSIDER OR RECOMMEND TO THE APPLICATION REVIEW SUBCOMMITTEE THAT THEY CONSIDER WHATEVER HAS BEEN SUBMITTED THUS FAR AND PAUSE ON ACCEPTING OTHERS MOVING FORWARD SO THAT THE HARD WORK PEOPLE HAVE PUT IN HAVE GONE THROUGH REVIEW IS CONSIDERED BY THE APPLICATION REVIEW SUBCOMMITTEE, BUT OTHER APPLICATIONS PERHAPS WE PAUSE ON THEM. THAT COULD BE SOMETHING --

DR. LEVITT: I UNDERSTAND THE RATIONALE.

I DON'T DISAGREE WITH A PAUSE SINCE WE ARE TRYING REALLY HARD TO DEVELOP A POLICY. BUT THERE NEEDS TO BE SOME COMMUNICATION WITH THE SCIENTIFIC COMMUNITY. AND THEN THAT MEANS YOU HAVE TO DEFINE WHAT YOU MEAN BY N OF 1. RIGHT? THERE ARE LOTS OF DISEASES THAT ARE DEFINED AS RARE, RIGHT, WHICH ARE NOT NECESSARILY N OF 1. SO I'M NOT A PROPONENT OF USING THE WORD "RARE." AND I THINK IF -- I'M NOT AND EXPERT IN THIS. I'M JUST SAYING LIKE THERE ARE A LOT OF PEDIATRIC DISEASES THAT ARE DEFINED AS RARE. AND SO DO THEY ALL GET PUT ON HOLD BECAUSE THEY'RE RARE? AND THE POPULATION MAY BE IN ANY PARTICULAR YEAR TEN NATIONALLY OR SOMETHING LIKE THAT.

SO I'M JUST CONFUSED ABOUT HOW TO DEFINE THIS. AND MAYBE THERE ARE EXPERTS HERE THAT CAN SPEAK TO THIS AND WHAT YOUR RECOMMENDATION IS TO THE LEADERSHIP TO HOW YOU WOULD DEFINE IT BECAUSE SOMEBODY HAS GOT TO DEFINE THE PAUSE. WE ARE ACCEPTING OR NOT -- WE'RE PAUSING ON ACCEPTING APPLICATIONS THAT HAVE X NUMBER OF SUBJECTS, RIGHT, THAT WOULD BE THE CLINICAL TRIAL, SOMETHING LIKE THAT.

DR. CREASEY: IF I CAN SAY SOMETHING, PAT. THE FDA SET UP A GUIDANCE DOCUMENT ON THE N OF 1. WE CAN FOLLOW THE GUIDANCE DOCUMENT. IT CLEARLY

DEFINES WHAT AN N OF 1 IS. AND SO IF WE CAN PUT THAT TOGETHER OR CAN SHARE IT WITH THE COMMITTEE FOR YOU TO LOOK AT IT AND IF IT SATISFIES BECAUSE NOT ALL RARE DISEASES ARE N OF 1. AND THIS IS WHY THE FDA HAS ACTUALLY CATEGORIZED N OF 1 AS ONE CATEGORY.

DR. LEVITT: OKAY. THAT WOULD BE HELPFUL, SURE. THAT'S GREAT. YEAH.

MR. FISCHER-COLBRIE: JUDY. J.T. SORRY. YOU WERE AHEAD, SO GO AHEAD, J.T.

DR. THOMAS: I WOULD JUST LIKE TO FOLLOW AGAIN WHAT MARIA SAID. I THINK THAT GIVEN THAT WE HAVE ONE APPLICATION WE'RE DEALING WITH HERE AND NOT CURRENTLY AN INFLUX OF MANY, THAT WOULD MAKE IT DIFFICULT TO PROCEED WITHOUT A POLICY IN PLACE, THAT WE EVALUATE THIS ONE IN THE ORDINARY COURSE. AND THEN HAVE THE LT GET BACK TO THE SCIENCE SUBCOMMITTEE ON ALL THESE DEFINITIONAL ISSUES THAT WILL INFORM ANY SORT OF PAUSE THAT MIGHT BE RECOMMENDED GOING FORWARD.

DR. CREASEY: I JUST WANT TO ALSO MENTION THAT THE WORKSHOP, THERE WAS A RECOMMENDATION FOR A PILOT FOR AN N OF 1 THAT INCLUDES SEVERAL OTHER TECHNOLOGIES. I JUST WOULD LIKE YOU TO MAKE SURE YOU'RE AWARE OF ALL THAT. THE FDA ITSELF ALREADY AGREES TO THE FACT THAT MAKING ALL THESE

TECHNOLOGIES AVAILABLE TO THE N OF 1 IS SOMETHING WE NEED -- THE WHOLE FIELD NEEDS TO RECOGNIZE.

MR. FISCHER-COLBRIE: JUDY.

DR. GASSON: I WOULD LIKE TO SUPPORT THE PAUSE OF CONSIDERATION OF N OF 1 TRIALS AS DEFINED BY THE FDA BASED UPON THE DISCUSSION SO FAR AND THE AMOUNT OF WORK THAT'S INVOLVED IN PREPARING, SUBMITTING, AND REVIEWING THESE APPLICATIONS.

MR. FISCHER-COLBRIE: MARIA, YOUR HAND IS UP. I DON'T KNOW IF YOU ALREADY COMMENTED OR NOT.

VICE CHAIR BONNEVILLE: I WAS GOING TO COMMENT THE SAME AS JUDY. SO THANK YOU.

MR. FISCHER-COLBRIE: SO IT SOUNDS LIKE THE QUESTION ON THE TABLE IS INHERENTLY THE RECOMMENDATION TO PROVIDE WITH RESPECT TO WHAT MIGHT BE DONE WITH AN APPLICATION OR APPLICATIONS IN THE PROCESS FROM A RECOMMENDATION PERSPECTIVE. AND SO THEN I'VE HEARD A COUPLE DIFFERENT VIEWPOINTS ON WHETHER TO ALLOW THAT TO GO FORWARD. KEITH.

DR. YAMAMOTO: I DON'T DISAGREE WITH THIS RECOMMENDATION TO PAUSE ON THE N OF 1S AS DEFINED BY THE FDA. BUT I DON'T KNOW HOW THE FDA DEFINES THE N OF 1. AS PAT HAS ALREADY POINTED OUT, IF IT REALLY IS N OF 1, WE HAVE THE SAME -- THE SAME ISSUES ARISE WHEN IT'S N OF 10 OR N OF 50. AND SO I DON'T WANT

TO BE TOO RESTRICTIVE ABOUT THIS. I THINK SETTING THE POLICY IF -- SETTING A PAUSE FOR THE CURRENT N OF 1 THAT FOLLOWS THE FDA RECOMMENDATION IS A FINE THING. I THINK THAT THE WORKING GROUP THAT'S GOING TO DEVELOP A RECOMMENDATION FOR POLICY IS GOING TO HAVE TO CONSIDER THESE RARE DISEASES MORE BROADLY THAN N OF 1 BECAUSE THE IMPLICATIONS FOR THE APPROPRIATION OF CIRM FUNDS FOR THESE STUDIES ARE VERY EXTENSIVE DOWNSTREAM AS WE'VE ALREADY EXPERIENCED, SORT OF ORCHARD THERAPEUTICS.

QUESTIONS ARISE WHETHER THIS IS THE RIGHT USE OF CIRM FUNDS. I'VE SAID IN PREVIOUS MEETINGS THAT I THINK IT IS, BUT I THINK THIS IS AN IMPORTANT DEBATE TO HAVE. SO I DON'T WANT TO EXTEND THIS ANY MORE THAN SIMPLY TO SAY THAT -- JUST REMIND US ALL THAT THE POLICY RECOMMENDATION THAT WE EVENTUALLY ARRIVE AT IS GOING TO EXTEND -- NECESSARILY WILL HAVE TO EXTEND TO STUDIES THAT ARE MORE THAN N OF 1 IF THE FDA IS DEFINING THIS IN THAT HIGHLY RESTRICTIVE MODE OF REALLY BEING A NUMBER OF REALLY BEING ONE.

DR. THOMAS: KEITH, THAT'S PRECISELY THE SCOPE OF THIS DISCUSSION. IT'S GOING TO GO BEYOND N OF 1 MORE BROADLY TO WHAT WE'LL CALL RARE DISEASE FOR THE MOMENT. SO WE WILL BE GETTING BACK WITH A

SORT OF BROADER VIEW OF THE ISSUE.

DR. YAMAMOTO: GREAT.

MR. FISCHER-COLBRIE: IT SOUNDS LIKE THE PROPOSAL ON THE TABLE CURRENTLY, THEN, IS TO, AGAIN, I'VE HEARD A COUPLE OF COUNTERVAILING VIEWS, BUT DELAY THE CURRENT APPLICATION OR MAKE A RECOMMENDATION NOT TO DELAY THE APPLICATION, BUT MAKE A RECOMMENDATION THAT WE GET MORE INFORMATION AROUND STRATEGY AND APPROACHES AROUND N OF 1 SPECIFICALLY IN ORDER TO CONSIDER FURTHERANCE OF APPLICATIONS. SO ABLA.

DR. CREASEY: I JUST WANT TO MENTION TO ALSO KEITH THAT AN N OF 1 IS ACTUALLY A SYNONYM OF INDIVIDUALIZED THERAPY. SO EACH PATIENT WILL GET THEIR OWN DRUG. THAT'S WHAT THE FDA ALSO SUPPORTS IN THEIR DESCRIPTION OF THE GUIDANCE. SO EVERY PATIENT WILL HAVE THEIR OWN ASO OR THEIR OWN CRISPR OR THEIR OWN WHATEVER TECHNOLOGY PLATFORM THAT IS BEING USED. THAT DRUG WILL BE ONLY FOR THE BENEFIT OF THAT ONE PATIENT. SO INDIVIDUALIZED.

MR. TOCHER: SORRY TO INTERRUPT. I WAS GOING TO SUGGEST WHEN THE TIME IS READY, I CAN MAKE A STAB AT STATING WHAT I THINK THE MOTION IS AS YOU HAVE STATED IT WHEN TIME IS APPROPRIATE.

MR. FISCHER-COLBRIE: I THINK IT WOULD BE

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GOOD TO DO THAT NOW FOR CLARIFICATION BECAUSE WE'VE HAD A COUPLE OF COMMENTS ALONG THE WAY HERE. SO JUST TO BE CRYSTAL CLEAR, I THINK IT WOULD BE GREAT TO STATE IT.

MR. TOCHER: THE MOTION WOULD BE, IF MADE, WOULD BE TO PAUSE ACCEPTING N OF 1 APPLICATIONS AS THAT TERM IS DEFINED BY FDA GUIDANCE UNTIL SUCH TIME AS THE TEAM CAN BRING TO THE BOARD A BROADER GUIDANCE ON THE ISSUE OF N OF 1, BUT TO ALLOW THE CURRENT APPLICATION UNDER CONSIDERATION BY THE APPLICATION REVIEW SUBCOMMITTEE TO CONTINUE TO BE CONSIDERED AT THE JANUARY 25TH MEETING.

MR. FISCHER-COLBRIE: OKAY. OTHER COMMENTS ABOUT THAT? IF NOT, WE CAN ENTERTAIN -- VITO.

DR. IMBASCIANI: THANK YOU. I WANT TO FOLLOW UP ON WHAT ABLA JUST SAID, THAT REFINING THE DEFINITION OF N EQUALS 1 TO IDENTIFY THE CREATION OF A PATIENT-SPECIFIC INDIVIDUAL MEDICATION. SO FOR ME, WHO'S NOT INVOLVED IN CLINICAL TRIALS, THIS DISTINCTION BETWEEN A SCIENTIFIC EXPERIMENT AND A CLINICAL THERAPY. SO I MAKE THIS COMMENT FOR THE BENEFIT OF THE LEADERSHIP TEAM, THAT WHEN THEY CONVENE TO DISCUSS THIS CONCEPT, COULD THEY KEEP THAT DISTINCTION IN MIND AND MAYBE SHED MORE LIGHT

ON THAT TO HELP THOSE OF US ON THE BOARD MAKE A DECISION ONE WAY OR THE OTHER AS TO WHETHER WE'RE GOING TO SUPPORT N OF 1 AS A SCIENTIFIC EXPERIMENT OR AS A CLINICAL THERAPY.

MR. FISCHER-COLBRIE: GREAT COMMENT. AND THAT WAS ALSO IN ONE OF SUPPORTING LETTERS IN THE CONTEXT THAT THIS APPROACH HAS OTHER POTENTIAL BENEFITS AS A CONSEQUENCE.

DR. IMBASCIANI: I'D LIKE TO COMPLIMENT THE AUTHOR OF THAT LETTER FOR REALLY STATING THAT DISTINCTION VERY CLEARLY. HE HELPED EDUCATE ME. THANK YOU.

MR. FISCHER-COLBRIE: OKAY. SO THERE'S A TENTATIVE MOTION ON THE TABLE AS OUTLINED BY SCOTT. KEITH, YOUR HAND WAS RAISED. IF YOU COULD GO AHEAD.

DR. YAMAMOTO: JUST A QUICK COMMENT. I SUPPORT THE MOTION FOR A PAUSE. BUT JUST TO REMIND US ALL THAT THAT FDA DEFINITION, VERY INTERESTING, I THINK IS VERY REASONABLE, IS THAT THE POPULATION OF PATIENTS THAT WILL QUALIFY AS N OF 1 IS GOING TO EXPAND RAPIDLY AS WE BETTER UNDERSTAND MECHANISMS OF DISEASE. AND THAT AS WE UNDERSTAND THOSE MECHANISMS, WE WILL FIND IT'S SOMETHING THAT WAS EMBEDDED IN A MESSAGE THAT I GAVE IN A TALK TO THE RARE DISEASE FOUNDATION SOME YEARS AGO WHERE I

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POINTED OUT THAT PEOPLE WORKING ON RARE DISEASES ARE REALLY AHEAD OF THE GAME BECAUSE, AS WE BETTER UNDERSTAND MECHANISMS OF DISEASE, PRECISION MEDICINE MOVES FORWARD. ALL DISEASE WILL BECOME RARE DISEASES. AND THEY WILL ALL BE DISTINGUISHED SPECIFICALLY BY THE COMBINATION OF FACTORS THAT COME TOGETHER THAT GIVE RISE TO THE DISEASE IN A GIVEN INDIVIDUAL. HOW MANY OF THOSE WILL BE TREATABLE BY SOME OF THE CURRENT TECHNOLOGIES OBVIOUSLY LESS CLEAR, BUT IT'S GOING TO INCREASE.

SO THIS POLICY DECISION THAT WE'RE GOING TO BE COMING TO, IF WE USE THE FDA DEFINITION, WE JUST NEED TO BE AWARE THAT IT'S GOING TO BE AN EXPANDING GROUP THAT WILL QUALIFY UNDER THOSE CONDITIONS.

MR. FISCHER-COLBRIE: THANK YOU, KEITH. WITH THAT, CAN I ENTERTAIN -- GET A CONSIDERATION FOR A -- FOR THE MOTION AND A SECOND?

VICE CHAIR BONNEVILLE: SO MOVED.

DR. IMBASCIANI: I'LL SECOND.

MR. FISCHER-COLBRIE: AND THEN WE OPEN IT UP FOR PUBLIC COMMENT; IS THAT CORRECT?

MR. TOCHER: WE CAN OPEN IT UP ACTUALLY FOR BOARD COMMENT. AND THEN AFTER BOARD COMMENT, WE'LL MOVE TO PUBLIC COMMENT.

MR. FISCHER-COLBRIE: THANK YOU. SO BOARD COMMENT ON THE MOTION? NOT SEEING ANY. GIVEN THAT, WE CAN OPEN UP FOR PUBLIC COMMENT.

DR. GLEESON: I APOLOGIZE FIRST. I DIDN'T MEAN TO SPEAK EARLIER. SO MY NAME IS JOSEPH GLEESON. I'M A PROFESSOR AT THE UNIVERSITY OF CALIFORNIA SAN DIEGO AND RADY CHILDREN'S HOSPITAL AND ALSO INVOLVED IN A CALIFORNIA-BASED NON-PROFIT NAMED N-LOREM, WHICH WAS FOUNDED BY THE FORMER -- THE FOUNDER OF IONIS PHARMACEUTICAL, A PUBLICLY TRADED COMPANY AND THE LEAD IN CREATING ANTISENSE OLIGONUCLEOTIDES FOR A RANGE OF MEDICAL CONDITIONS.

I'VE BEEN REALLY -- IT'S REALLY AN HONOR TO SPEAK HERE TODAY OR ADDRESS YOU. AND I'VE GOT A REALLY GOOD PERSPECTIVE ON WHERE THIS GROUP IS COMING FROM.

I JUST WANT TO CLARIFY A COUPLE THINGS, AND I'LL BE HAPPY TO ANY ANSWER QUESTIONS. FIRST, THIS GRANT ALREADY -- THIS IS A GRANT FROM OLIVIA KIM-MCMANUS, WHO'S ACTUALLY ON THE CALL TODAY, A COLLEAGUE OF MINE AT CHILD NEUROLOGY DIVISION AT RADY CHILDREN'S. AND IT ALREADY UNDERWENT REVIEW BY ARS AND RECEIVED A PERFECT SCORE, INCLUDING A GREAT DIVERSITY SCORE. IT WAS QUITE WELL ADDRESSED THE MISSION OF CIRM, WHICH IS ACCELERATE WORLD-CLASS

SCIENCE TO DELIVER TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND WORLD.

THE APPLICATION MET ALL THE CRITERIA. OLIVIA HOLDS AN IND. SHE HAS A DRUG. SHE HAS A PATIENT READY TO BE DOSED. SO I THINK THE QUESTION THAT I'M HEARING THAT'S KIND OF CRYSTALLIZING FROM THE COMMITTEE IS WHAT IS AN N OF 1? IS IT A TRIAL OR IS IT A THERAPY? AND THE WAY I THINK ABOUT IT IT'S ACTUALLY TWO SIDES OF THE SAME COIN. IT IS A TRIAL, AND THE PATIENTS ARE RECEIVING A DRUG. AND AT THE END OF THE YEAR, WE LEARN IF THE DRUG WORKED OR NOT BECAUSE THERE ARE QUANTITATIVE OUTCOME MEASURES.

THE ADDITIONAL BENEFIT IS THAT THE PATIENT CAN IMPROVE POTENTIALLY. AND, OF COURSE, THAT'S ALSO THE CASE IN STANDARD CLINICAL TRIALS. ONLY HALF THE PATIENTS GENERALLY RECEIVE THE ACTIVE COMPOUND.

AND THESE ARE CALLED N OF 1S, BUT REALLY THE FDA CALLS THEM N OF FEW. IF WE HAVE A DRUG, IF ANYONE HAS A DRUG THAT CAN BE USED IN LESS THAN 30 INDIVIDUALS, IT CAN FALL UNDER THIS SPECIAL FDA CONSIDERATION. AND ALTHOUGH WE CALL THIS PATIENT N OF 1, THERE'S ALMOST A THOUSAND PATIENTS THAT HAVE

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SCN2A MUTATIONS. THIS IS NOT A RARE DISEASE. IT'S A RARE DISEASE AS DEFINED BY THE NIH, LESS THAN 200,000, BUT IT IS NOT AN N OF 1 DISEASE. THESE SAME ASO'S, IF THEY PROVE POSITIVE IN ONE PATIENT, WILL OPEN UP A WHOLE WORLD OF SCIENCE IN THE FUTURE THAT WILL ALLOW US TO BOTH ADMINISTER THIS SAME DRUG TO OTHER PATIENTS AS LONG AS THERE'S UNDER 30, AS WELL AS WE CREATE OTHER ANTISENSE DRUGS THAT ARE SPECIFIC FOR OTHER MUTATIONS.

CALIFORNIA IS IN AN AMAZING POSITION HERE BECAUSE WE HAVE CIRM. WE HAVE N-LOREM HERE BASED IN CALIFORNIA. AND WE HAVE A NUMBER OF FANTASTIC GENOMICS ORGANIZATIONS THAT ARE LEADING THE CHARGE IN DIAGNOSIS THAT ARE JUST GOING TO CREATE THIS WAVE OF NEW DISCOVERY AND OPPORTUNITY FOR NEW DRUGS AND IMPROVING THE LIVES OF CALIFORNIANS.

MS. MANDAC: WE'RE AT TIME, DR. GLEESON.

DR. GLEESON: THANK YOU.

MR. FISCHER-COLBRIE: OTHER PUBLIC COMMENT? DR. MIGNON.

DR. MIGNON: HI. THANK YOU VERY MUCH, DR. FISCHER-COLBIRE.

MY NAME IS DR. MIGNON, AND I LEAD CLINICAL DEVELOPMENT AT THE N-LOREM FOUNDATION, LIKE JOE SAID, A CALIFORNIA-BASED NON-PROFIT. I'M ALSO BASED

IN CALIFORNIA MYSELF.

I DID SUBMIT A LETTER THAT I WILL NOT READ RIGHT NOW BECAUSE I JUST ALSO WANT TO ADDRESS A COUPLE OF POINTS FROM WITHIN THE LETTER, BUT ALSO ADDRESS SOME OF THE QUESTIONS THAT WERE POSED DURING THE DISCUSSION RIGHT NOW.

SO FIRST I JUST WANTED TO SAY THESE ARE REALLY TRULY UNDERREPRESENTED PATIENTS. AND SO FINANCIAL MEANS DOES NOT COME INTO PLAY WHEN A PATIENT SUBMITS THE APPLICATION TO N-LOREM. WE WILL DEVELOP THE MEDICINE FOR THEM FOR FREE, AND WE'LL PROVIDE IT TO THEM FOR LIFE IF THE MEDICINE SEEMS TO WORK IN THESE PATIENTS. SO I THINK THIS IS VERY IMPORTANT.

SO IN TERMS OF DIVERSITY AND EQUITY, WE WILL PROVIDE THE DRUG TO ANYBODY WHO WILL COME AND SUBMIT AN APPLICATION AND WHO ACTUALLY IS ACCEPTED, MEANING THAT THE MUTATION CAN BE TREATED WITH AN ASO AND WE BELIEVE THAT WE CAN HAVE BENEFIT.

I ALSO WANTED TO MENTION THAT EVEN THOUGH WE TALK ABOUT AN N OF 1, THE N OF 1 OF TODAY WILL CERTAINLY BECOME THE N OF MANY TOMORROW, ESPECIALLY WITH MORE GENOME SEQUENCING BEING DONE AS DR. YAMAMOTO WAS SAYING. SO EVEN THOUGH WE MAY THINK OF THIS AS WE'RE ONLY FOCUSING ON ONE PATIENT AND WHAT

IS THE BENEFIT TO THAT, I THINK THE BENEFIT IS THAT WE HAVE A HUGE POSSIBILITY OF UNDERSTANDING BIOLOGY AND MECHANISM OF ACTION AND MECHANISM OF DISEASE. WE ARE REALLY TARGETING THE MOLECULAR MECHANISM OF DISEASE OF THESE PATIENTS. AND SO BY UNDERSTANDING THAT BIOLOGY, WE'LL BE ABLE TO APPLY THIS BIOLOGY FROM THIS DISEASE TO MANY MORE DISEASES AND TO COMPLEX DISEASES AND SO FORTH.

SO I THINK WE SHOULD NOT JUST THINK OF IT AS THAT WE'RE ONLY TREATING ONE PATIENT. AND TO GIVE YOU AN EXAMPLE, OUR FIRST PATIENT WE STARTED TO TREAT A YEAR AGO THAT HAS A KIF1A MUTATION, A VERY SPECIFIC KIF1A MUTATION. WE DEVELOPED A SELECTIVE ASO FOR THAT PATIENT. HER SEIZURES HAVE DRAMATICALLY DROPPED. SHE HAS BEEN ABLE TO GAIN IN DOMAINS THAT WE DIDN'T THINK SHE COULD GAIN AGAIN. AND WE HAVE ALSO FOUND THAT THAT SPECIFIC ASO, EVEN THOUGH IT WAS SPECIFICALLY DEVELOPED FOR HER BASED ON A SPECIFIC SNP IN HER GENOME, WE'LL ABLE TO USE IN OTHER PATIENTS WITH KIF1A.

SO I THINK WE THINK WE WILL LEARN OVER TIME THAT, AGAIN, WE START TREATING ONE PATIENT, BUT WE'LL BE ABLE TO TREAT MORE PATIENS AND REALLY UNDERSTAND HOW WE CAN AFFECT THE GENETIC MUTATION.

SO ALSO WANTED TO SAY THAT THE FDA IS

UPDATING THEIR GUIDANCE DOCUMENTS, AND WE'VE HAD REALLY GOOD DISCUSSIONS WITH THEM OF SPECIFICALLY WHAT HAPPENS TO THE N OF A FEW. AND SO I THINK THIS IS A ROADMAP THAT IS BEING DEVELOPED. BUT IN THE MEANTIME I THINK IT'S VERY IMPORTANT FOR US TO FOCUS ON THE PATIENTS AT HAND AND PATIENTS IN FRONT OF US AND THE PATIENTS THAT DR. KIM-MCMANUS IS TRYING TO TREAT THAT HAS AN ASO DEVELOPED FOR THEM, AND WE NEED FUNDING TO BE ABLE TO START THE CLINICAL TRIAL. SO THIS KID IS JUST WAITING FOR TREATMENT TO START. AND BECAUSE WE STARTED TREATING ANOTHER SCN2A PATIENT AT ANOTHER UNIVERSITY, WE KNOW THAT SUCH THERAPY AND TARGETING THE MOLECULAR BASIS OF THIS IS WORKING BECAUSE IT'S WORKING IN THIS OTHER PERSON, ALSO AGAIN REDUCING THEIR SEIZURES.

SO WITH THAT, I JUST WANTED TO BRING OUT THOSE POINTS AND JUST LET YOU KNOW WHERE WE THINK THE FUTURE IS, THAT WE'LL BE ABLE TO REALLY UNDERSTAND DISEASE BIOLOGY, MECHANISM.

MR. TOCHER: THANK YOU, DR. MIGNON. YOUR THREE MINUTES IS UP. I KNOW WE WANT TO GET TO THE OTHERS WHO HAVE THEIR HANDS RAISED. THANK YOU.

MR. FISCHER-COLBRIE: I SEE DR. WILLIFORD.

DR. WILLIFORD: YES. DR. AMY WILLIFORD. I ALSO WORK WITH DR. MIGNON AT THE N-LOREM

FOUNDATION.

I JUST WANT TO REITERATE SOME POINTS THAT SHE HAD MADE AND ALSO JUST KIND OF PROVIDE A LITTLE BIT OF UNDERSTANDING ON HOW THE SPECIFIC PROPOSAL THAT WE HAVE ON THE TABLE COULD REALLY HELP BRIDGE THE GAP BETWEEN WHAT WE'RE DOING HERE A N-LOREM AS A DRUG DISCOVERY AND NON-PROFIT DRUG DISCOVERY GROUP TO SUPPORT THE PHYSICIANS WHO ARE HELPING THESE PATIENTS, WHO HAVE THESE PATIENTS COME IN AND THEY NEED SUPPORT TO HELP WITH THE CLINICAL COST OF CARE.

AND SO THERE WAS A COUPLE OF THINGS THAT I THINK, WHEN YOU'RE THINKING ABOUT N OF 1, WHICH WE THINK OF AS N OF FEW, I THINK AS LAURY MENTIONED, THAT WE HAVE FOUND OTHER PATIENTS THAT CAN BENEFIT FROM DRUGS THAT WE HAVE DEVELOPED. AND I THINK AS WE GET MORE AND MORE DRUGS DEVELOPED FOR A SINGLE PATIENT, WE'RE GOING TO FIND MORE AND MORE PATIENTS THAT COULD BENEFIT FROM THAT SINGLE DRUG.

AND SO WHAT WE'RE REALLY LOOKING FOR IS A WAY TO SUPPORT THE PHYSICIANS THAT ARE WILLING TO TREAT THESE PATIENTS THAT HAVE WORKED WITH US IN THE DEVELOPMENT, THE DISCOVERY, WORKED WITH US IN IDENTIFYING MEASURES THAT WE CAN LOOK AT HOW TO ASSESS THE BENEFIT OF THESE DRUGS AND SUPPORTING THESE PHYSICIANS AND THEIR PATIENTS.

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I THINK WE'RE IN THE EARLY DAYS OF UNDERSTANDING HOW SINGLE-GENE MUTATIONS CAN AFFECT SORT OF THE OVERALL HEALTH OF THE PATIENT. AND WHAT WE LEARNED FROM OUR KIF1A PATIENTS AND OUR SCN2A PATIENTS WILL HELP THAT ENTIRE GROUP OF PATIENTS WHETHER THEY HAVE THAT SPECIFIC GENE MUTATION OR NOT. WE THINK THAT THESE EXPERIMENTS ARE REALLY IMPORTANT TO PROVIDE BROADER UNDERSTANDING FOR THAT DISEASE AND WILL HELP ALL OF THOSE PATIENTS.

AND THEN I JUST WANTED TO ALSO REITERATE HOW UNDERSERVED THESE PATIENTS ARE. AND SO IN MY ROLE, I DEAL WITH A LOT OF THE PATIENTS THAT COME INTO N-LOREM. AND THESE PATIENTS ARE KIND OF AT THE END OF THEIR ROAD. AND SO THEY'VE HAD A LONG DIAGNOSTIC JOURNEY. THEY'VE HAD NO HELP FROM THE HEALTHCARE INDUSTRY ASIDE FROM GETTING A DIAGNOSIS AND UNDERSTANDING THAT THERE'S NO MEDICINE THAT CAN HELP THESE PATIENTS. AND SO THEY REALLY ARE, WHEN YOU THINK ABOUT AN UNDERSERVED PATIENT POPULATION, THEY ARE THE MOST UNDERSERVED POPULATION THAT WE HAVE. A LOT OF THEM DO NOT HAVE THE FINANCIAL MEANS TO PAY FOR THEIR CLINICAL CARE.

AND SO I THINK IT'S A HEALTHCARE RESPONSIBILITY THAT WE HELP THESE PATIENTS EVEN THOUGH THEY'RE NOT A PATIENT THAT WOULD HAVE, LIKE,

DIABETES OR A VERY LARGE DISEASE. THEY CAN STILL BE ABLE TO ACCESS THE CARE THAT THEY NEED AND THE MEDICINES THAT THEY NEED TO HAVE A QUALITY OF LIFE.

MR. FISCHER-COLBRIE: CLAUDETTE, I DON'T KNOW IF THERE ARE ANY OTHER PUBLIC COMMENT. I DON'T SEE ANY. IF YOU SEE ANY THERE.

DR. KIM-MCMANUS: WILL I BE ABLE TO SPEAK AT THIS POINT? I'M SO SORRY. I DIDN'T RAISE MY HAND. MY NAME IS OLIVIA KIM-MCMANUS. I'M THE PI ON THE GRANT THAT WAS SUBMITTED, THE SINGLE APPLICATION THAT IS PROMPTING THIS DISCUSSION.

JUST AS BACKGROUND, I DO WORK AT UC SAN DIEGO, RADY CHILDREN'S HOSPITAL IN ASSOCIATION WITH THE CHILDREN'S INSTITUTE OF GENOMIC MEDICINE. I APPRECIATE THE SUPPORT AND THE EFFORT THAT IS GIVEN TO MY N OF 1 APPLICATION. ULTIMATELY, LIKE DR. YAMAMOTO SAID, THESE ARE NOT REALLY N OF 1 DISEASES, AND THERE ARE GOING TO BE MORE DISEASES THAT ARE DIAGNOSED IN THE FUTURE IN CALIFORNIA. SPECIFICALLY NEURODEVELOPMENTAL DISEASE IMPACTS MORE THAN ONE OUT 50 CALIFORNIANS WITH EPILEPSY AND AUTISM AND MORE THAN 75,000 CHILDREN AT BIRTH THROUGH TWO, TWO DOZEN YEARS AGO WERE ELIGIBLE FOR EARLY INTERVENTION AND MANY OF THESE ARE BIOGENETIC ETIOLOGIES. WHEN THESE GENETIC THERAPIES AS THEY ARE COMING DOWN THE

PIPELINE, WHETHER THROUGH ASO OR GENE REPLACEMENT, AND THEY ARE TAILORED TO THE ETIOLOGY OF THOSE SYMPTOMS, THIS IS GOING TO BE VERY IMPORTANT IN IMPROVING THE CLINICAL CARE FOR THESE PATIENTS, BUT ALSO FOR US TO HELP UNDERSTAND THE PATHOPHYSIOLOGY OF GENETIC DISEASE SO THAT WE CAN OPTIMIZE (UNINTELLIGIBLE) BETTER SCIENCE AS WELL.

AND SO THESE KIDS WITH DEVELOPMENTAL EPILEPTIC ENCEPHALOPATHIES DUE TO GENETIC MUTATIONS ARE ACTUALLY MORE COMMON THAN SMA; BUT BECAUSE OF THE HETEROGENEITY, THERE'S NO ONE COMMON TREATMENT OR COHESIVE EFFORT. AND SO THIS IS EXACTLY WHY THESE PATIENTS WITH RARE MUTATIONS ARE AN UNDERSERVED DEMOGRAPHIC THAT SPECIFICALLY SPEAKS TO CIRM'S MISSION FOR SUPPORT BECAUSE THEY ARE UNABLE TO GET ANY SORT OF COMMERCIAL EFFORTS TOWARDS DRUG DEVELOPMENT BECAUSE OF THE RARITY OF DISEASE.

THE QUESTION IS NOT REALLY ABOUT WHETHER SHOULD WE SUPPORT THIS MISNOMERED, PERHAPS, N OF 1, AND WE REFER TO THAT CURRENTLY, BUT HOPEFULLY IN THE FUTURE, AS THESE OTHER THERAPIES COMING DOWN THE PIPELINE PROMPT MORE AND MORE GENETIC TESTING, EARLIER DIAGNOSIS, AND POTENTIAL FOR INTERVENTIONAL THERAPIES, WE WILL LEARN WHETHER OR NOT BECAUSE THIS IS A SCIENTIFIC EXPERIMENT. IT'S NOT A CLINICAL

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THERAPY OR TREATMENT. THIS IS A SCIENCE AND THIS IS WHAT WE'RE LOOKING AT IN TERMS OF ONE PATIENT AS A PHASE 1 OR PHASE 2 THAT WE CAN'T DO IN A STANDARD CLINICAL TRIAL FORMAT, BUT THAT HAS VALIDITY AND RELEVANCE FOR NOT JUST THIS ONE DISEASE, BUT FOR MANY GENETIC DISEASES BECAUSE THERE ARE SO MANY THAT ARE JUST NOT TESTED.

THE QUESTION IS NOT WHETHER WE DELIVER IT TO THIS ONE PATIENT, BUT HOW WILL THIS FURTHER OUR OPPORTUNITY TO GET MORE GENETIC TESTING AVAILABLE ACROSS THE BOARD FOR ALL CALIFORNIANS BECAUSE THERE, FOR SURE, ARE OTHER PATIENTS WHO ARE NOT DIAGNOSED AND NEED TO HAVE ACCESS TO GENETIC THERAPY AND DIAGNOSIS, NOT JUST THE THERAPY, BUT DIAGNOSIS. AND SO THIS IS WHERE CIRM IS UNIQUELY POSITIONED. I AGREE THAT CIRM SHOULD NOT -- IT DOESN'T HARM THE CIRM MANDATE TO FUND EVERY SINGLE TRIAL OUT THERE, BUT WHEN THERE IS --

MR. TOCHER: DR. MCMANUS, TIME IS UP I'M AFRAID.

MR. FISCHER-COLBRIE: OKAY. OTHER PUBLIC COMMENT, QUESTIONS?

MS. MANDAC: THERE ARE NO HANDS RAISED VIRTUAL OR IN PERSON.

MR. TOCHER: I THINK WE'RE READY TO

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PROCEED TO A VOTE WHEN YOU ARE.

MR. FISCHER-COLBRIE: YES. LET'S PROCEED WITH THE VOTE. ROLL CALL PLEASE.

MR. TOCHER: HAIFAA ABDULHAQ.

DR. ABDULHAQ: YES.

MR. TOCHER: MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: DEBORAH DEAS.

DR. DEAS: YES.

MR. TOCHER: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YES.

MR. TOCHER: JUDY GASSON.

DR. GASSON: YES.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: YES.

MR. TOCHER: PAT LEVITT.

DR. LEVITT: YES.

MR. TOCHER: CHRIS MIASKOWSKI.

DR. MIASKOWSKI: YES.

MR. TOCHER: KAROL WATSON.

DR. WATSON: YES.

MR. TOCHER: KEITH YAMAMOTO.

DR. YAMAMOTO: YES.

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MR. TOCHER: THANK YOU VERY MUCH. THE MOTION CARRIES. MARK.

MR. FISCHER-COLBRIE: OKAY. THAT WRAPS UP THE DISCUSSION FOR THIS POINT, AND I'LL TURN IT OVER TO THE RIGHT PERSON TO CLOSE THE MEETING OUT.

MR. TOCHER: AT THIS POINT WE'LL CALL FOR PUBLIC COMMENT ON ANY MATTER NOT ON THE AGENDA. SO, CLAUDETTE, CAN YOU LET US KNOW IF ANYONE ELSE HAS FURTHER COMMENT?

MS. MANDAC: THERE ARE NO HANDS RAISED VIRTUAL OR PHYSICAL.

MR. TOCHER: GREAT. THANK YOU. MARK AND LARRY, YOU CAN ADJOURN THE MEETING IF YOU HAVE NO FURTHER COMMENTS.

MR. FISCHER-COLBRIE: NONE FOR ME. LARRY, I DON'T KNOW IF YOU HAVE OTHER COMMENTS TO WRAP UP.

DR. GOLDSTEIN: NO. LET'S CLOSE IT UP.

(THE MEETING WAS THEN CONCLUDED AT 9:58 A.M.)

133 HENNA COURT, SANDPOINT, IDAHO 83864
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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 JANUARY 17, 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.

BETH C. DRAIN, CA CSR 7152
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