BEFORE THE
ACCESS 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 8, 2026
9:30 A.M.

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

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MAY 8, 2026; 9:30 A.M.

CHAIRPERSON BONNEVILLE: GREAT. GOOD MORNING, EVERYONE. THANK YOU FOR ATTENDING TODAY'S ACCESS AND AFFORDABILITY WORKING GROUP MEETING. I'D LIKE TO CALL THIS MEETING TO ORDER. AND, CAMERON, CAN YOU PLEASE TAKE THE ROLL?

MR. MALIK: ABSOLUTELY.

MARIA BONNEVILLE.

CHAIRPERSON BONNEVILLE: PRESENT.

MR. MALIK: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: PRESENT.

MR. MALIK: ADRIANA PADILLA IS NOT PRESENT. TED GOLDSTEIN, ALSO NOT PRESENT. AMMAR QADAN.

DR. QADAN: PRESENT.

MR. MALIK: JAMES DEBENEDETTI.

MR. DEBENEDETTI: HERE.

MR. MALIK: MAHESWARI SENTHIL, NOT PRESENT. ADRIENNE SHAPIRO, NOT PRESENT. HARLAN LEVINE.

DR. LEVINE: RIGHT HERE.

MR. MALIK: THANK YOU. PAT LEVITT, NOT PRESENT. DARIUS LAKDAWALLA.

DR. LAKDAWALLA: HERE.

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MR. MALIK: THANK YOU. CHRISTINA HARTMAN.

MS. HARTMAN: PRESENT.

MR. MALIK: THANK YOU. KIM BARRETT.

DR. BARRETT: PRESENT.

MR. MALIK: THANK YOU. LIZ BOILEAU. SHE TOLD ME SHE WOULD JOIN LATER. AND YAEL WYTE. OH, IT LOOKS LIKE PAT JUST JOINED.

DR. LEVITT: SORRY I'M LATE.

MR. MALIK: NO PROBLEM. ALL RIGHT.

CHAIRPERSON BONNEVILLE: GREAT. THANK YOU. THERE ARE NO VOTING ITEMS TODAY. THESE ARE ALL INFORMATIONAL.

I WANT TO START BY INTRODUCING DR. SHYAM PATEL. HE'S THE NEW ASSOCIATE VICE PRESIDENT OF PATIENT ACCESS. AND SOME OF YOU MAY ALREADY KNOW HIM, BUT FOR THOSE OF YOU WHO DON'T, SHYAM HAS BEEN AT CIRM FOR QUITE SOME TIME AND HAS HELD VARIOUS ROLES IN HIS TENURE. AND MOST RECENTLY, HE WAS THE ASSOCIATE VICE PRESIDENT OF PRECLINICAL DEVELOPMENT. AND WE ARE VERY EXCITED TO HAVE HIM IN THIS NEW ROLE.

OUR FIRST AGENDA ITEM IS A RECAP OF A MEETING CIRM ORGANIZED AND CO-HOSTED IN MARCH ALONG WITH ISSCR AND UCLA. THE MEETING BROUGHT TOGETHER ACADEMICS, INCLUDING SEVERAL OF OUR OWN GRANTEES,

ECONOMISTS, MANUFACTURERS, AND POLICYMAKERS. AND SO, SHYAM, CAN YOU WALK US THROUGH SOME OF THOSE KEY TAKEAWAYS?

DR. PATEL: OF COURSE. AND THANK YOU FOR THE INTRODUCTION, MARIA.

AND SO JUST AS A LITTLE BIT OF BACKGROUND, AS THE PATIENT ACCESS TEAM WORKS WITH MARIA TO GO THROUGH THE TRANSITION AND TO ONBOARD NEW MEMBERS, WE'LL BE WORKING WITH ALL THE AAWG MEMBERS GOING FORWARD. AND TODAY WE'RE FOCUSING ON GIVING YOU SOME UPDATES ON SOME OF THE BROADER ENGAGEMENT THAT CIRM HAS BEEN PARTICIPATING IN ON THIS TOPIC OF ACCESS AND AFFORDABILITY FOR CELL AND GENE THERAPIES AS WELL AS A COUPLE OF OPERATIONAL UPDATES. ALL OF THIS IS TO SET UP BROADER THE INTERACTIONS WITH THE AAWG GOING FORWARD IN YOUR PARTNERSHIP ON VARIOUS ITEMS THAT WE WANT TO EXECUTE ON ON OUR ACCESS AND AFFORDABILITY STRATEGY. SO THIS WAS ONE KEY COMPONENT OF THAT OUTREACH THAT WE WERE DOING AND JUST BROADER ENGAGEMENT WITH DIFFERENT STAKEHOLDERS.

SO MARIA WAS INSTRUMENTAL IN CO-ORGANIZING THIS EVENT WITH THE UCLA AND ISSCR TEAMS. AND SEVERAL AAWG MEMBERS ATTENDED THIS MEETING, AND WE INVITE THEM TO SPEAK UP IN JUST A SECOND. SO THIS IS A SUMMIT FOCUSING ON TOPICS THAT ARE WELL WORN IN

THE CELL AND GENE THERAPY FIELD AROUND HOW DO WE MAKE THESE THERAPIES MORE ACCESSIBLE? HOW DO WE PAY FOR THEM? AND WHAT ARE THE DIFFERENT STAKEHOLDERS' CONSIDERATIONS, INCLUDING ACADEMIC INVESTIGATORS, CLINICIANS, HEALTHCARE PROVIDERS, DRUG DEVELOPERS, DRUG MANUFACTURERS, ALL THE DIFFERENT PAYERS, AS WELL AS GOVERNMENT FUNDING AGENCIES.

AND THERE THE SIMILAR THEMES THAT WERE DISCUSSED PREVIOUSLY: PAYER PARTICIPATION, EMERGING POLICY AND AFFORDABILITY MODELS, AS WELL AS MANUFACTURING AND REGULATORY INNOVATION, WHICH TENDS TO BE VERY MUCH UPSTREAM OF COMMERCIAL PRODUCTS, BUT DOES DRAMATICALLY IMPACT HOW THOSE PRODUCTS ARE MADE AVAILABLE TO PATIENTS.

SOME OF THE KEY THEMES ARE IN THE NEXT SLIDE, AND I'M GOING TO BRIEFLY DISCUSS THESE, AND THEN I'M GOING TO TURN IT OVER AND ASK SOME OF THE AAWG MEMBERS WHO ATTENDED TO SPEAK TO THEM AS WELL. NEXT SLIDE, PLEASE, CAMERON.

SO AS WE ALL KNOW, FROM A MACROECONOMIC PERSPECTIVE, THERE ARE SIGNIFICANT BENEFITS OF HAVING THESE ONCE AND DONE CELL AND GENE THERAPIES THAT COULD POTENTIALLY BE CURATIVE OR AT MINIMUM BEING DISEASE-MODIFYING. HOWEVER, IT'S PRETTY CLEAR THAT THAT MACROECONOMIC ARGUMENT DOESN'T ALWAYS

FILTER DOWN TO THE INDIVIDUAL STAKEHOLDERS' ECONOMIC INCENTIVES AND BENEFITS OR EVEN AT THE PATIENT LEVEL.

AND SO, IN THAT SENSE, BEING ABLE TO DELIVER, PAY FOR, AND PROVIDE THESE THERAPIES TO THE PATIENTS WHO NEED THEM WILL REQUIRE EVOLUTION OF OUR SYSTEMS. AND THAT WAS A KEY THEME THAT WAS BROUGHT UP OVER AND OVER AGAIN. AND IN MANY INSTANCES SOME OF THE PAYERS NOTED THAT THERE ARE VARIOUS OTHER CONSIDERATIONS THAT THEY'RE THINKING ABOUT. BUT THEY RECOGNIZE, GIVEN THE LARGE PIPELINE OF CELL AND GENE THERAPIES THAT ARE IN DEVELOPMENT, THAT THIS IS SOMETHING THAT NEEDS TO BE ADDRESSED AT A BROADER LEVEL THAN ON INDIVIDUAL THERAPIES.

ON THE THERAPY DEVELOPER SIDE, THIS IS SOMETHING THAT WE'VE WORKED WITH YOU TO STRESS IN OUR AWARDS AS WELL. BUT GIVEN THE NATURE AND THE COMPLEXITIES OF DELIVERING AND PAYING FOR CELL AND GENE THERAPIES, DEVELOPERS NEED TO START PLANNING EARLY. A LOT OF IT IS RESEARCH EARLY ON FOLLOWED BY MORE DIRECT PLANNING FOR EVIDENCE GENERATION AND STAKEHOLDER ENGAGEMENT AS THEY GO THROUGH LATER CLINICAL DEVELOPMENT. AND SO THIS WAS REINFORCED SEVERAL TIMES. AND ALSO IT WAS A POINT OF DISCUSSION BETWEEN ACADEMICS, THERAPY DEVELOPERS,

AND THE VARIOUS STAKEHOLDERS ON HOW THIS COULD BE DONE MORE EFFECTIVELY GOING FORWARD.

AND LASTLY, MANUFACTURING INNOVATION IS ESSENTIAL TO LONG-TERM AFFORDABILITY AND ACCESS, PARTICULARLY, NOT ONLY AROUND REDUCING COGS, BUT ALSO JUST MAKING IT MORE SCALABLE. ONE KEY AREA WAS WHEN WE LOOK AT REGENERATIVE MEDICINE CELL THERAPIES, THESE ARE DERIVED FROM PLURIPOTENT STEM CELLS, AND THEY'RE USUALLY TARGETING BROADER INDICATIONS WHERE BEING ABLE TO MANUFACTURE THESE THERAPIES AT SCALE AND BEING ABLE TO DELIVER THEM TO PATIENTS IS GOING TO BE CRITICAL TO MEET THE NEEDS OF THOSE DISEASE INDICATIONS IN THOSE PATIENTS.

SO I'M GOING TO PAUSE THERE, AND I'M GOING TO ASK DR. DARIUS LAKDAWALLA, DR. HARLAN LEVINE, DR. KIM BARRETT, AND MARIA AND J.T. IF YOU WANT TO ADD ANYTHING. YOU WERE AT THIS SUMMIT. MANY OF YOU SPOKE AS WELL. AND SO APPRECIATE ANY FEEDBACK AND ANY PERSPECTIVES YOU CAN PROVIDE.

CHAIRPERSON BONNEVILLE: KIM.

DR. BARRETT: YEAH. I WAS REALLY EXCITED TO BE AT THE SUMMIT. I THOUGHT IT WAS AN EXTREMELY WELL-ORGANIZED AND VERY WORTHWHILE DAY. IT'S CERTAINLY WORTH MY TIME TO BE THERE. IT WAS EXTREMELY EDUCATIONAL.

I THINK ONE OF MY KEY TAKEAWAYS WAS THAT THERE ARE SOME REALLY SMART SOCIAL SCIENTISTS OUT THERE THINKING ABOUT THESE ISSUES. AND I THINK I SHOULD HAVE LOOKED UP THE NAME OF THE PERSON THAT GAVE AN EARLY, SORT OF, KEYNOTE ON THE PAYMENT MODELS AND THE ECONOMIC ASPECTS OF --

DR. LEVINE: DANA GOLDMAN?

DR. BARRETT: YEAH. BUT THAT WAS A REALLY COMPELLING TALK FOR ME. AND THIS IDEA THAT OUR SORT OF MODEL FOR HEALTH INSURANCE DOES NOT ALIGN WITH THESE LIFELONG CURATIVE THERAPIES IS REALLY ONE THAT I THINK HAS TO BE ADDRESSED VERY AGGRESSIVELY. I THINK IT'S SOMETHING THAT CIRM SHOULD BE INVESTING IN THAT TYPE OF ECONOMIC RESEARCH.

AND PROBABLY WHAT WE NEED IS SOME SORT OF AMORTIZATION OF THE COSTS OF THESE THERAPIES ACROSS THE HOPEFULLY SUBSTANTIALLY EXTENDED LIFE EXPECTANCY FOR PEOPLE WHO WILL RECEIVE THESE CURATIVE TREATMENTS.

THE OTHER TAKEAWAY FOR ME WAS THAT THIS PROBLEM ABSOLUTELY HAS TO BE SOLVED OR IT WILL REALLY BEGIN TO STIFLE INNOVATION AND, MORE IMPORTANTLY, INVESTMENT IN THE SECTOR.

AND THEN FINALLY, I JUST COMPLETELY SECOND THE COMMENTS ON MANUFACTURING. THAT WAS, FOR ME,

ALSO A VERY INFORMATIVE AND USEFUL PANEL.

CHAIRPERSON BONNEVILLE: THANK YOU. J.T.

DR. THOMAS: MORNING, EVERYBODY. SO ONE OF THE BIGGEST TAKEAWAYS, SORT OF A MACRO POINT FROM MY PERSPECTIVE, WAS THE HIGHLY UNUSUAL COMBINATION OF EXPERTISE THAT WAS GATHERED IN THE ROOM. THIS WAS A SESSION THAT WAS REALLY UNLIKE ANY THAT I THINK ANY OF US AT CIRM HAVE HAD IN BRINGING ALL THE DISPARATE STAKEHOLDERS TOGETHER TO TALK ABOUT THESE VARIOUS THEMES THAT SHYAM AND KIM HAVE BEEN DESCRIBING.

AND SO BOTH IN THE PRESENTATIONS AND THE PANEL DISCUSSIONS AND THE SIDEBARS, WHICH THESE SORTS OF THINGS ARE ALWAYS REALLY IMPORTANT, THE VALUE OF CONVENING THESE DIFFERENT STAKEHOLDER GROUPS BECAME EVEN MORE EVIDENT THAN WE KNEW IT WAS GOING TO BE GOING INTO IT. AND I KNOW THAT MARIA AND THE ISSCR FOLKS, MARIA AND TEAM, WENT TO GREAT LENGTHS TO CREATE THIS HYBRID SORT OF EVENT. AND I THINK THAT IT SETS THE TABLE FOR TAKING GREAT ADVANTAGE OF THE NETWORKING THAT CAME OUT OF IT TO ADDRESS THE VARIOUS ISSUES THAT WERE ON THE TABLE.

SO THAT WAS, FOR ME, THE SINGLE GREATEST VALUE OF WHAT WAS A VERY SUCCESSFUL DAY BY ALL ACCOUNTS OF EVERYBODY THERE.

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CHAIRPERSON BONNEVILLE: THANK YOU, J.T. DARIUS.

DR. LAKDAWALLA: THANKS. SO SHYAM DID A GREAT JOB OF SUMMARIZING IT. SO I'LL BE QUICK ABOUT IT. ONE THING I TOOK AWAY AS AN ECONOMIST WAS HOW LITTLE WE HAVE INNOVATED VERSUS THE WAY THE BASIC SCIENTISTS HAVE INNOVATED IN THIS AREA, AND THAT ECONOMIC POLICY AND HEALTH POLICY NEED TO DO A LOT OF WORK TO CATCH UP. THAT WAS NO. 1.

NO. 2, I WAS ACTUALLY QUITE SURPRISED AT THE WIDESPREAD AGREEMENT ON A LOT OF THE ISSUES THAT NEED TO BE SOLVED. AND THAT'S NOT ALWAYS SO COMMON IN HEALTH POLICY CONTEXT. I THINK PEOPLE AGREE THAT THE REIMBURSEMENT MODEL, IT NEEDS TO BE ABLE TO ACCOUNT FOR OUTCOMES, IT NEEDS TO BE ABLE TO ACCOUNT FOR THE MISMATCH BETWEEN THE TIMING OF PAYMENT AND THE ACCRUAL OF BENEFITS, AND IT NEEDS TO DEAL WITH THE FACT THAT OUR PRICE NEGOTIATION MODEL TODAY IS PREDICATED ON DENIAL OF ACCESS, WHICH IS A REAL PROBLEM WHEN YOU'RE TREATING RARE DISEASE WITH NOVEL CELL AND GENE THERAPIES. SO THAT WAS HEARTENING.

ONE THING THAT WAS LESS HEARTENING TO ME WAS I THINK THERE WAS APPROPRIATE SKEPTICISM ABOUT THE EXTENT TO WHICH POLICY COULD BE CHANGED TO ACCOMMODATE THESE SORTS OF INNOVATIONS, WHICH MEANS

THAT THIS IS A PROBLEM THAT WE NEED TO, AND PROBABLY CAN, MAKE PROGRESS AGAINST IN THE MARKETPLACE TO DEVELOP NEW MODELS FOR FINANCIAL INTERMEDIATION. IN PARTICULAR, THAT WAS A PROMISING AREA OF DISCUSSION.

SO I CAME AWAY WITH A FAIR AMOUNT OF CAUTIOUS OPTIMISM AND ALSO MORE FOCUS IN WHAT I THINK WE ARE -- WHAT THE TASK LIES -- WHAT THE TASK IS THAT LIES AHEAD OF US FROM THE STANDPOINT OF HEALTH ECONOMICS AND HEALTH POLICY.

CHAIRPERSON BONNEVILLE: THANK YOU, DARIUS.

AND I WANT TO HIGHLIGHT, BOTH DARIUS AND HARLAN PRESENTED AND WERE SPEAKERS AT THE MEETING. SO THANK YOU. AND DARIUS WAS PART OF THE ORGANIZING COMMITTEE AS WELL.

CHRISTINA.

MS. HARTMAN: THANKS, MARIA. SORRY I WASN'T ABLE TO JOIN THE MEETING. IT SOUNDS LIKE IT WAS VERY SUBSTANTIVE.

JUST A COUPLE THINGS FROM MY PERSPECTIVE IN WASHINGTON. AND AS A RARE PARENT, A COUPLE THINGS THAT HAVE COME UP RECENTLY, WE'RE SEEING A LOT OF COMPANIES THAT WANT TO FOCUS ON MORE COMMON DISORDERS. AND THEY'RE LOOKING AT BEING ABLE TO

FUND THEIR COMMERCIAL ENTERPRISES THROUGH THERAPIES FOR COMMON DISORDERS. BUT MEANWHILE, THOSE OF US WHO REALLY WANT TO FOCUS ON PEDIATRIC RARE DISEASE IN PARTICULAR, THAT REMAINS A SIGNIFICANT CHALLENGE.

AND ONE OF THE CONCERNS I HAVE IS WHEN YOU LOOK AT THE POTENTIAL PROFITABILITY OF DIFFERENT ASSETS, MOST COMPANIES ARE CONCERNED WITH THAT PURE PROFITABILITY. HOW MUCH CAN WE MAXIMIZE PROFITABILITY? AND WHAT ASSET IS GOING TO TAKE US TO THE TOP OF THAT RANGE? WE NEED TO SOMEHOW GET TO A PLACE WHERE WE'RE CONSIDERING MAKING SURE THERE ARE ASSETS IN THE RARE SPACE THAT CAN BE PROFITABLE. THEY WON'T BE LIKE A GLP-1 LEVEL OF PROFITABILITY, BUT THEY CAN STILL BE A PROFITABLE ASSET. AND COMPANIES THAT BELIEVE THAT EQUALLY IMPORTANT TO PROFITABILITY IS THE VALUE YOU DELIVER TO SOCIETY.

HAVING THAT BALANCE, I THINK, IS GOING TO BE IMPERATIVE. AND IF WE CAN SOMEHOW GET BACK TO THAT, THAT'S REALLY IMPORTANT BECAUSE THE INCENTIVES ARE VERY DIFFERENT FOR RARE PEDIATRIC DISEASE. AND THEY WILL NEVER BE WHAT YOU SEE FOR COMMON DISORDERS. SO SOMEHOW MAKING SURE THAT THAT'S A FOCUS.

THE OTHER TWO BIG THINGS I'VE SEEN IN WASHINGTON RECENTLY IS THERE'S A LOT OF DISCUSSION

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ABOUT ACADEMIC VERSUS COMMERCIAL COMPANY MODELS. THERE DOES NEED TO BE A MORE IN-DEPTH DISCUSSION, I THINK, ABOUT WHAT CAN ACADEMIC MODELS ACCOMPLISH AND WHAT ARE THE LIMITATIONS ON THAT VERSUS WHAT CAN YOU DO COMMERCIALLY, PARTICULARLY WHEN YOU'RE THINKING ABOUT DIFFERENT SIZED POPULATIONS AND THE ABILITY TO SCALE.

AT ORCHARD WE HAVE A RARE PEDIATRIC GENE THERAPY, LENMELDY, THAT WE EXPECT TO SEE 40 OR 50 BIRTHS A YEAR IN THE UNITED STATES THAT WOULD BE ADDRESSABLE WITH OUR GENE THERAPY IF WE CAN FIND THOSE KIDS IN TIME. SO THAT'S THE MAXIMUM RANGE FOR OUR THERAPY.

UNFORTUNATELY, THERE ARE NOT A LOT OF COMPANIES OUT THERE THAT ARE TRYING TO COMMERCIALIZE RARE PEDIATRIC GENE THERAPIES FOR THESE SMALLER POPULATIONS. WE'VE SEEN RECENTLY A LOT OF ATTENTION FROM THE CURRENT ADMINISTRATION ON BABY KJ AND THAT N OF 1 MODEL, WHICH IS VERY EXCITING, BUT, AGAIN, VERY LIMITED IN TERMS OF WHAT WE CAN DO, AT LEAST IN THE PRESENT DAY OR IN THE VERY NEAR FUTURE.

WE'VE ALSO SEEN A LOT OF DISCUSSION ABOUT THE LARGER RARE DISEASES, LIKE SICKLE CELL. WE HAVE THAT CENTERS FOR MEDICARE AND MEDICAID INNOVATION CELL AND GENE THERAPY MODEL, THE DEMO, WHICH AGAIN,

THAT'S GREAT WHEN YOU HAVE COMPETITORS IN A SPACE, LIKE YOU HAVE THE LYFGENIA AND CASGEVY COMPETITORS. OTHER RARE DISEASES DON'T HAVE COMPETITORS. LIKE WITH LENMELDY, WE ARE THE ONLY THERAPY OUT THERE FOR METACHROMATIC LEUKODYSTROPHY.

SO THE QUESTION REALLY I THINK WE'RE LOOKING AT IN WASHINGTON IS WE'VE GOT A LOT OF FOCUS AND ATTENTION AND POLICY ON THESE N OF 1S, LIKE BABY KJ. WE'VE GOT A LOT OF ATTENTION AND FOCUS ON POLICY FOR THESE LARGER RARE POPULATIONS LIKE THE 100,000 OR SO PEOPLE IN AMERICA WITH SICKLE CELL DISEASE. BUT THERE'S REALLY NOTHING FOR THOSE TENS AND HUNDREDS, THOSE RARE DISEASES WITH TENS AND HUNDREDS OF PEOPLE. AND SO HOW DO WE SOMEHOW FOCUS ON THAT AND INCENTIVIZE THAT AS WE'RE WORKING ON THE N OF 1S AND WORKING ON THE MUCH LARGER RARE DISEASE POPULATIONS? AND THEN WHERE'S THAT SWEET SPOT WITH ACADEMIC VERSUS COMMERCIAL? BECAUSE I THINK THAT THERE'S ROOM FOR EVERYTHING. AND AS WE KNOW, WITH 10,000 OR SO RARE DISORDERS, AS WE'VE DISCOVERED WITH GENOMIC SEQUENCING, WE'VE ONLY GOT TREATMENTS FOR ABOUT 5 PERCENT OF THEM. SO LONG WAY TO GO.

CHAIRPERSON BONNEVILLE: THANK YOU, CHRISTINA. THAT WAS AMAZING. THANK YOU.

DOES ANYONE HAVE ANY OTHER COMMENTS?

DR. LEVINE: IT'S HARD TO ADD TO THE SUMMARY THAT HAD PROCEEDED, BUT I WOULD JUST SAY I AGREE THAT DANA GOLDMAN'S PRESENTATION IS REALLY INTERESTING. I HAD A SIDEBAR WITH HIM, AND HE TALKED ABOUT THE LIFE VALUE, WHICH IS NOT JUST MEDICAL SAVINGS, BUT PRODUCTIVITY TO SOCIETY, IS ABOUT $300,000 PER CANCER PATIENT SAVED.

SO IN RARE DISEASES, THE NUMBERS WERE LIKE IN THE 150,000 TO $200,000 RANGE BASED ON SOME STUDIES. SO I MEAN REALLY INTERESTING DATA.

IN MY PANEL WHAT I TALKED ABOUT WERE THEMES THAT WE TALKED ABOUT HERE, OR I'VE TALKED ABOUT HERE BEFORE, WHICH IS THIS SYSTEM, PARTICULARLY IN CALIFORNIA, AS WE FORM THESE TIGHT MEDICAL GROUPS TO DELIVER COST-EFFECTIVE CARE, WE OFTEN ARE LEAVING OUT THE EXPERTISE THAT PEOPLE UNDERSTAND RARE CONDITIONS OR UNDERSTAND THE NEWER TREATMENTS IN CANCER. AND THERE'S JUST LESS REFERRAL IN FOR PROPER TESTING AND THEN FOR PROPER TREATMENT.

AND THEN ALSO THE FACT THAT I THINK SEVERAL HAVE MENTIONED THAT AS MEDICAL GROUPS ARE BEING ASKED TO TAKE ON MEDICAL RISK, THERE'S TOO STRONG INCENTIVE TO NOT WANT TO TEST AND REFER TO GET PEOPLE TO GET THESE TREATMENTS. SO WE HAVE TO

KIND OF FIX THOSE SYSTEMS. AND WE'RE BEGINNING TO SEE DIFFERENT COMPANIES FORMING TO TRY TO AMORTIZE THE COST THAT WE TALKED ABOUT.

I THINK THE COMMENT WAS MADE THAT THE SCIENCE IS AHEAD OF THE SOCIOLOGIC RESPONSE TO IT, OR THE BUSINESS RESPONSE, AND I THINK YOU'RE GOING TO SEE THE SAME WAY THAT PEOPLE HAVE CARVED OUT, LIKE, CATASTROPHIC ILLNESS -- CATASTROPHIC THINGS LIKE EARTHQUAKES AND FIRES AND HURRICANES. I THINK PEOPLE ARE GOING TO START TO CARVE OUT CATASTROPHIC DISEASE AND TRY TO CREATE A DIFFERENT INSURANCE MECHANISM FOR THAT. BUT WE'RE STILL EARLY DAYS FOR SAYING THAT, BUT I THINK IT'S GOING TO BE REALLY IMPORTANT BECAUSE THE WAY WE MANAGE RISK TODAY REALLY IS NOT CONDUCIVE TO A FREE FLOW OF PATIENTS INTO THESE SORTS OF TREATMENTS.

CHAIRPERSON BONNEVILLE: AGREE. THANK YOU FOR THAT.

I THINK THE CONFERENCE OR THE SYMPOSIUM, THE DAY FOR ME WAS, IT WAS GREAT TO SEE EVERYONE, AS J.T. SAID, EVERYONE IN A ROOM TOGETHER AND TALKING ABOUT THESE ISSUES. AND I THINK THE MORE WE CAN CONVENE PEOPLE IN SITUATIONS LIKE THIS TO HAVE CONVERSATIONS, I THINK EVERYONE WALKS AWAY LOOKING AT IT FROM SOMEONE ELSE'S POINT OF VIEW. AND I

THINK THAT THAT'S REALLY IMPORTANT BECAUSE A LOT OF THE TIMES WE JUST GET VERY FOCUSED ON OUR OWN AREA OF EXPERTISE OR OUR OWN AREA THAT WE LIVE DAY TO DAY. AND I THINK BRINGING EVERYONE IN A ROOM TOGETHER TO UNDERSTAND THE CHALLENGES IS VERY IMPORTANT.

SO THANK YOU, EVERYONE WHO WAS THERE. AND THANKS, SHYAM, FOR THIS OVERVIEW.

DR. PATEL: THANK YOU, MARIA. AND THANK YOU, EVERYONE, FOR YOUR GREAT INPUT AND PERSPECTIVES.

SO WE'LL MOVE ON TO THE NEXT FEW AGENDA ITEMS.

CHAIRPERSON BONNEVILLE: THANK YOU.

DR. PATEL: THANK YOU, MARIA.

SO THE NEXT ITEM HERE IS THE FDA WORKSHOP. SO THIS WAS RELATED TO SOME OF WHAT CHRISTINA WAS TALKING ABOUT WITH RESPECT TO PEDIATRIC RARE DISEASES. SO WE HAVE A RICH PORTFOLIO AT CIRM OF PEDIATRIC RARE GENETIC DISEASE THERAPIES, PARTICULARLY IN THE IN VIVO GENE THERAPY SIDE.

AND ONE OF THE KEY QUESTIONS THERE THAT THE FDA HAD RAISED WAS -- TO THE COMMUNITY WAS DO WE NEED TO RETHINK HOW CLINICAL TRIALS ARE GENERALLY DONE FOR RARE PEDIATRIC DISEASE CELL AND GENE

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THERAPY TREATMENTS? IN PARTICULAR, AS MANY OF YOU KNOW, A FIRST-IN-HUMAN TRIAL OFTEN WILL START IN ADULT POPULATIONS TO DEMONSTRATE SAFETY BEFORE MOVING ON TO A PEDIATRIC PATIENT POPULATION. AND FOR CELL AND GENE THERAPIES, THAT IS NOT ALWAYS THE APPROPRIATE ETHICAL OR SCIENTIFIC APPROACH BECAUSE THOSE DISEASES ARE UNIQUE TO CHILDREN.

OFTENTIMES THE THERAPY HAS AN EFFICACY DOSE THAT IS PROVIDED TO THOSE PATIENTS. AND SO IN THAT RESPECT, HAVING THIS CONVERSATION, IT WAS REALLY IMPORTANT.

THE OTHER PART THAT THIS WORKSHOP ALSO ADDRESSED WAS AT WHAT POINT TO TREAT PATIENTS. IS IT PRE-SYMPTOMATIC? IS IT EARLY SYMPTOMATIC? IS IT LATE SYMPTOMATIC? AND REALLY, SORT OF, HOW TO TARGET YOUR THERAPIES TO THE DISEASE PROFILE.

AND SO THIS PARTICULAR WORKSHOP, THE FDA WORKSHOP WAS HOSTED AT THE FDA CAMPUS, AND IT WAS CO-SPONSORED BY CIRM, AND IT WAS ORGANIZED BY THE ALLIANCE FOR REGENERATIVE MEDICINE. IT BROUGHT TOGETHER VARIOUS STAKEHOLDERS, INCLUDING CLINICIANS, THE FDA LEADERSHIP, PARENTS, PATIENT ADVOCATES, AND THE INDUSTRY.

AND I WILL TELL YOU THAT THE MOST FORCEFUL AND MOST IMPACTFUL VOICE WAS COMING FROM THE PARENTS

THEMSELVES, PARTICULARLY ON BOTH OF THE TOPICS THAT I JUST MENTIONED. AND THERE WERE VARIOUS PATIENT ACCESS CONSIDERATIONS THAT WERE BEING BROUGHT UP.

SO ON THE TOPIC OF DO YOU DESIGN A CLINICAL TRIAL FOR PEDIATRIC PATIENTS FIRST, OR DO YOU FIRST TEST IT IN ADULTS, THERE WAS A VERY RICH DISCUSSION AROUND THIS, PARTICULARLY AROUND WHAT EVIDENCE IS NEEDED AND HOW TO STRUCTURE THAT TRIAL. AND THE PATIENT ADVOCATES AND THE PATIENT FAMILIES REPEATEDLY REINFORCED THE FACT THAT PARENTS AND PATIENT ADVOCATES MAKE RISK-BENEFIT DECISIONS ALL THE TIME.

AND HERE THEY CAN BE APPROPRIATELY INFORMED TO MAKE RISK-BENEFIT DECISIONS ON ENROLLING IN CLINICAL TRIALS. AND THEY STRESS REPEATEDLY THAT IT'S THE RESPONSIBILITY OF CLINICIANS, THERAPY DEVELOPERS, AND REGULATORS TO ACTIVELY SEEK INPUT IN THE DESIGN AND EXECUTION OF THESE TRIALS. RATHER THAN TO JUST SIMPLY FOCUS ON PATIENT UNDERSTANDING AND CONSENT, BRING THEM INTO THE PROCESS, ENGAGE THEM ON THE TRIAL DESIGN AND EXECUTION. AND THAT WAS A POINT THAT WAS REPEATEDLY ENFORCED OVER AND OVER AGAIN. AND I THINK THE FDA AND THE DEVELOPERS NOW HAVE THE INCENTIVE AND CLEAR MANDATE TO IMPROVE HOW CLINICAL TRIALS ARE DESIGNED FOR PEDIATRIC RARE

DISEASE TREATMENTS.

ON THE OTHER ELEMENT, THERE WAS A RICH DISCUSSION AROUND WHEN TO TARGET YOUR TREATMENT BECAUSE A LOT OF THESE DISEASES ARE PROGRESSIVE. AND SO DO YOU TREAT PRE-SYMPTOMATIC, IN THE EARLY ONSET OF SYMPTOMS, OR DO YOU WAIT UNTIL YOU HAVE A REALLY CLEAR, SORT OF COURSE OF ILLNESS TO TREAT BECAUSE THESE ARE HIGH-RISK, HIGH-BENEFIT THERAPIES.

AND I THINK, IN THAT RESPECT, THE PATIENT ACCESS COMPONENTS THAT WERE COMING UP WERE REALLY FASCINATING. SO IF WE THINK ABOUT IT FROM, IF THE FOCUS IS REALLY TO TREAT EARLY TO GET THE MAXIMUM CLINICAL BENEFIT, YOU'RE LEAVING OUT PATIENTS WHO COULD POTENTIALLY BENEFIT LATER IN THE COURSE OF THE DISEASE. AND THIS IS SOMETHING THAT HAS PLAYED OUT IN CIRM CLINICAL TRIALS.

ON THE OTHER HAND, IF YOU ARE GOING TO FOCUS ON TREATING EARLY AND EVEN PRE-SYMPTOMATIC, YOU REALLY HAVE TO MAKE SURE THAT THERE'S EQUITABLE ACCESS TO DIAGNOSTIC AND SCREENING INFRASTRUCTURE FOR PATIENTS SO THAT THEY CAN BE APPROPRIATELY DIAGNOSED OR SCREENED AND ENROLLED IN THOSE TRIALS OR EVENTUALLY HAVE ACCESS TO THOSE THERAPIES.

SO ALL OF THOSE THEMES WERE EXPLORED OVER THE COURSE OF THE DAY. AND I THINK THE MAIN

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TAKEAWAY HERE WAS THAT THERE'S MUCH LEARNING, LISTENING, AND COORDINATION TO BE DONE BETWEEN ALL THE STAKEHOLDERS TO HELP ENSURE THAT SAFE AND EFFECTIVE CELL AND GENE THERAPIES ARE DEVELOPED AND MADE ACCESSIBLE TO CHILDREN.

CHAIRPERSON BONNEVILLE: THANK YOU, SHYAM.

DR. PATEL: I'LL MOVE ON.

CHAIRPERSON BONNEVILLE: DOES ANYONE HAVE ANY QUESTIONS OR COMMENTS FROM THE WORKSHOP?

DR. LEVINE: THE ONLY COMMENT I WOULD MAKE IS TO TIE IT TO WHAT I THOUGHT WAS ONE OF THE BEST QUESTIONS AT THE SESSION A COUPLE WEEKS AGO WAS A PEDIATRICIAN WAS SAYING SHOULDN'T THERE BE MORE EMPHASIS ON STARTING TREATMENT EARLIER BECAUSE IT SAVES MONEY TO INTERVENE QUICKER. LIKE YOU AVOID A LOT OF UNNECESSARY COSTS AND YOU KEEP THE PERSON HEALTHIER. AND OUR SYSTEM JUST ISN'T DESIGNED THAT WAY AT ALL. AND I THOUGHT IT WAS A REALLY INTERESTING QUESTION THAT, QUITE FRANKLY, I DIDN'T UNDERSTAND THE QUESTION THE WAY IT WAS WORDED; BUT AS I HEARD OTHERS RESPOND TO IT, I THOUGHT IT WAS VERY THOUGHT-PROVOKING.

CHAIRPERSON BONNEVILLE: THANK YOU. ANY OTHER QUESTIONS OR COMMENTS? OKAY. IF NOT, SHYAM, NEXT ITEM.

DR. PATEL: THANK YOU. SPEAKING OF PEDIATRIC GENE THERAPIES, SO CIRM, ON MARCH 26TH, WE HAD OUR FIRST APPROVED GENE THERAPY THAT CIRM HAD FUNDED. AND THIS WAS BY ROCKET PHARMA. THE FDA APPROVED ITS KRESLADI GENE THERAPY THROUGH THE ACCELERATED APPROVAL PATHWAY ON MARCH 26TH OF THIS YEAR. AND I'M GOING TO GO THROUGH A LITTLE BIT OF THE BACKGROUND OF THIS THERAPY, THE PATIENT POPULATION, CIRM'S ROLE IN IT, AS WELL AS FORWARD-LOOKING WHAT THE COMPANY PLANS TO DO.

SO FIRST OF ALL, THIS IS A GENE THERAPY TREATMENT FOR LEUKOCYTE ADHESION DEFICIENCY 1, WHICH IS AN INBORN ERROR OF IMMUNITY. BASICALLY IT'S A -- YOU HAVE A GENETIC MUTATION THAT PREVENTS THE IMMUNE SYSTEM FROM FUNCTIONING NORMALLY, AND IT RESULTS IN PATIENTS BEING VERY SUSCEPTIBLE TO INFECTION. AND IN THE MOST SEVERE FORMS OF THE DISEASE, YOU HAVE LIFE-THREATENING INFECTIONS EARLY IN CHILDHOOD AND IT'S OFTEN FATAL.

SO HERE SEVERE LAD1 PATIENTS ARE VERY SUSCEPTIBLE TO DISEASE BECAUSE THEY HAVE A MUTATION IN A GENE THAT IS NECESSARY FOR NORMAL NEUTROPHIL FUNCTION. AND SO WITHOUT THAT, THE IMMUNE SYSTEM CAN'T FIGHT OFF INFECTION, AND IT'S OFTEN FATAL FOR THESE PATIENTS WITHIN THE FIRST FEW YEARS OF THEIR

CHILDHOOD.

THE ONLY STANDARD OF CARE IS BONE MARROW TRANSPLANT. AND OFTENTIMES IT'S MATCHED-SIBLING DONOR TRANSPLANT, WHICH IS VERY DIFFICULT. AND SO IT'S IMPORTANT THAT, WHEN A PATIENT IS DIAGNOSED, THAT THEY'RE TREATED EARLY BECAUSE OF THE LIFE-THREATENING NATURE OF THIS DISEASE.

THIS PARTICULAR THERAPY IS A LENTIVIRAL GENE THERAPY. SO THE PATIENT'S BONE MARROW CELLS, BLOOD STEM CELLS, ARE HARVESTED. IN THE LAB THEY'RE GENETICALLY MODIFIED WITH A LENTIVIRAL VECTOR, AND THEN THEY'RE RE-INFUSED BACK INTO THE PATIENT WHERE THEY PRODUCE NORMAL NEUTROPHILS AND ARE ABLE TO FIGHT OFF INFECTIONS.

AND SO THIS PARTICULAR THERAPY WAS APPROVED BASED ON A VERY SMALL CLINICAL TRIAL BECAUSE THE PATIENT POPULATION IS VERY SMALL. THIS IS AN ULTRA-RARE DISEASE. ROCKET ESTIMATES IT'S GOING TO BE ABOUT 25 NEW CASES A YEAR. AND SO THEY WERE ABLE TO GET ACCELERATED APPROVAL WITH A NINE-PATIENT TRIAL. AND IT HAD MULTIPLE SITES ACROSS THE COUNTRY -- SORRY -- ACROSS THE WORLD, AND THE PRIMARY SITE WAS UCLA.

OUT OF THE NINE PATIENTS, SIX WERE TREATED AT UCLA WITH CIRM FUNDING. SO CIRM HAD FUNDED THE

UCLA PORTION OF THE TRIAL, AND MOST OF THE PATIENTS WERE TREATED AT THAT UCLA SITE BY DR. DON KOHN, WHO'S IN THE PICTURE HERE.

AND SO A COUPLE OF THE THINGS THAT I DO WANT TO HIGHLIGHT IS THAT THIS, FOR US, WAS AN EXAMPLE OF BOTH THE COMPANY AND THE CLINICAL SITE GOING ABOVE AND BEYOND TO MAKE SURE THAT PATIENTS AND THEIR FAMILIES WERE SUPPORTED THROUGH THE WHOLE JOURNEY OF DIAGNOSIS, TREATMENT, AND FOLLOW-UP.

SO TO GIVE YOU AN EXAMPLE, ONE OF THE FAMILIES THAT WAS ENROLLED IN THIS TRIAL, ALL THREE OF THE SIBLINGS WERE DIAGNOSED WITH LAD1. AND SO THAT FAMILY HAD TO TRAVEL TO L.A. ALL THREE SIBLINGS HAD TO BE TREATED, AND THEY HAD TO STAY IN L.A. FOR WEEKS FOR ALL THE FOLLOW-UP. AND SO BOTH THE UCLA TREATMENT SITE AS WELL AS ROCKET WORKED TOGETHER TO MAKE SURE THAT THEY COULD HANDLE THE LOGISTICS, THE HOUSING, AS WELL AS ALL OF THE TREATMENT FOR THOSE THREE KIDS.

THERE WERE ALSO A SIGNIFICANT PROPORTION OF INTERNATIONAL PATIENTS. AND IN THAT RESPECT, THE SPONSOR HAD TO ENSURE THAT THEY COULD QUICKLY ARRANGE FOR TRAVEL PLANS, ENTRY TO THE COUNTRY, AS WELL AS LANGUAGE SUPPORT WHEN THEY WERE HERE IN L.A. BEING TREATED AND FOLLOWED UP FOR THE THERAPY. AND

SO IN THAT INSTANCE, THIS IS AN EXAMPLE OF REALLY GOING ABOVE AND BEYOND FOR THIS PARTICULAR PATIENT POPULATION AND THEIR FAMILIES TO MAKE SURE THAT THEY HAD ACCESS TO THIS THERAPY IN THE CLINICAL TRIAL SETTING.

AFTER APPROVAL, ROCKET PLANS TO LAUNCH THIS PRODUCT AT THE END OF THIS YEAR. AND AS YOU KNOW, ALL CIRM-FUNDED THERAPIES THAT REACH THE APPROVAL STAGE ARE REQUIRED TO SUBMIT AN ACCESS PLAN TO CIRM WITHIN TEN DAYS OF BLA APPROVAL. IN THIS PARTICULAR INSTANCE, ROCKET HAS BEEN GRANTED AN EXTENSION TO PROVIDE THEIR ACCESS PLAN. IT'LL BE REVIEWED BY CIRM AND ALSO WILL BE AVAILABLE FOR PUBLIC COMMENT. AND WE EXPECT THAT THIS PARTICULAR PRODUCT WILL STAY ON ITS LAUNCH TRAJECTORY FOR THE END OF THIS YEAR.

CHAIRPERSON BONNEVILLE: THANK YOU, SHYAM. DOES ANYONE HAVE ANY COMMENTS OR QUESTIONS ABOUT THAT? IT'S VERY EXCITING NEWS, AS YOU CAN IMAGINE. SO I DON'T KNOW IF THERE'S ANY QUESTIONS ABOUT THE ACCESS PLAN OR ANYTHING SHYAM JUST MENTIONED.

DR. LEVITT: SHYAM, WHAT ARE THE FINANCIALS OF THIS, THE ESTIMATED FINANCIALS?

DR. PATEL: GOOD QUESTION. THEY HAVE NOT DISCLOSED PRICING. THEY WILL DO SO AS THEY GET

CLOSER TO A LAUNCH OF THE PRODUCT. SIMILAR THERAPIES, LENTIVIRAL GENE THERAPIES FOR OTHER IMMUNE DISORDERS, CHRISTINA CAN COMMENT ON THAT MUCH BETTER THAN I CAN.

DR. LEVITT: SO IS THE PLAN FOR THE HOSPITALS TO EAT THE DIFFERENCE BETWEEN REIMBURSEMENT AND THE PRICING?

DR. PATEL: SO IN THAT PARTICULAR CASE, IN TERMS OF REIMBURSEMENT, WHAT OUR UNDERSTANDING IS IS THAT FOR THESE ULTRA-RARE THERAPIES, THERE IS A REIMBURSEMENT PATHWAY FOR THEM FOR THE THERAPY. BUT, AGAIN, I WOULD DEFER TO CHRISTINA WHO MIGHT HAVE MORE COMMENTS ON THAT.

MS. HARTMAN: YEAH. I MEAN, I CAN TELL YOU MY EXPERIENCE WITH BLUEBIRD AND NOW ORCHARD. WHERE AT BLUEBIRD FOR LYFGENIA, THE SICKLE CELL GENE THERAPY THAT WE HAD, LENTIVIRAL, THE PATIENT POPULATION MIX WAS ABOUT 50 PERCENT MEDICAID -- I'M SORRY -- 50 PERCENT COMMERCIAL, 40 PERCENT MEDICAID, 10 PERCENT MEDICARE. MOST PEOPLE WEREN'T LIVING LONG ENOUGH TO BE ON MEDICARE AT ORCHARD.

AND JUST A QUICK COMMENT FROM THE DISCUSSION BEFORE. FOR MOST OF OUR KIDS, WE CAN'T TREAT THEM ONCE THEY'RE TOO SYMPTOMATIC. RIGHT? SO THE GENE THERAPY DOESN'T WORK IF THEY'RE TOO FAR

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PROGRESSED TO TREAT. SO THAT'S WHY YOU HAVE TO DIAGNOSE AND GET THEM EARLY, WHICH IS THE BIG CHALLENGE THERE. BUT AT ORCHARD, WE ARE ACTUALLY FINDING THAT IN PRACTICE, WE ARE SEEING ABOUT 70 PERCENT MEDICAID AND 30 PERCENT COMMERCIAL. WE HAD EXPECTED IT TO BE ABOUT A 50-50 SPLIT. IN PRACTICE, I THINK IN LARGE PART BECAUSE THESE KIDS ARE SO SICK, IT'S 70 PERCENT MEDICAID AND 30 PERCENT COMMERCIAL IS WHAT WE'RE CURRENTLY SEEING.

THE CHALLENGE THAT WE'VE HAD AT BOTH PLACES THAT I'VE BEEN CONSISTENTLY ARE THE DELAYS IN COVERAGE WHEN IT COMES TO MEDICAID. SO THE HOSPITALS NEED TO BE CONFIDENT THAT THEY'RE GOING TO BE REIMBURSED SEPARATELY FOR THE DRUG PRODUCT, BUT THEN ALSO FOR THE ANCILLARY CARE AND SERVICES. THE WAY THAT THESE AUTOLOGOUS EX VIVO LENTIVIRAL VECTOR GENE THERAPIES ARE BEING DELIVERED IS VIA A HEMATOPOIETIC STEM CELL TRANSPLANT. AND SO THAT HOSPITALIZATION, ALL THE ANCILLARY CARE AND SERVICES IS SIGNIFICANT, AND THE HOSPITALS NEED TO BE CONFIDENT THAT THEY'RE GOING TO RECEIVE THE REIMBURSEMENT FOR BOTH.

AS A COMPANY, WE ALSO HAVE TO BE CONFIDENT THAT WE ARE GOING TO BE ABLE TO PROCEED WITH THE MANUFACTURING. RIGHT? THE HOSPITAL HAS TO SIGN OFF

ON THAT PURCHASE ORDER BEFORE WE CAN PROCEED. WE ALSO HAVE LIMITED MANUFACTURING SPOTS. I MEAN, WE ARE MANUFACTURING AROUND THE GLOBE FOR LENMELDY IN THE U.S., LIBMELDY IN EUROPE. AND WE ALSO ARE RUNNING CLINICAL TRIALS FOR OTHER DISORDERS. SO THERE'S LIMITED MANUFACTURING SPACE. SO ALL OF THAT COMES INTO PLAY.

YOU'RE ALSO WORKING WITH THE PATIENT'S OWN CELLS. RIGHT? SO THERE'S A LIMITED AMOUNT OF THAT THERE AS WELL. BUT I DO THINK THE BIGGEST ISSUE THAT WE'VE SEEN, WHEN I WAS AT BLUEBIRD, WE SAW DELAYS OF 6 TO 24 MONTHS FOR MEDICAID PATIENTS. AT ORCHARD THAT 6- TO 24-MONTH DELAY WOULD BE DEATH. SO WE HAVE BEEN ABLE TO WORK SUCCESSFULLY WITH ELECTED OFFICIALS AND OTHERS TO MAKE SURE THAT THEY CAN EXPEDITE THE COVERAGE FOR THIS VERY SMALL NUMBER OF PATIENTS. BUT IT'S NOT SOMETHING THAT A COMPANY SHOULD HAVE TO DO, AND I DON'T THINK THAT IT IS FEASIBLE TO CONTINUE TO DO THAT IN THE LONG TERM. AND FOR COMPANIES THAT MAY NOT HAVE HAD THE INVESTMENT AND SOPHISTICATION AND BACKING THAT WE'VE HAD, IT MAY NOT BE POSSIBLE.

SO IT'S NOT A GREAT SITUATION, AND I THINK THERE'S A LOT THAT NEEDS TO EVOLVE THERE.

DR. LEVITT: YEAH. WELL, I COULDN'T AGREE

MORE. AND IT'S THE POSTER CHILD FOR SCIENCE WAY AHEAD OF POLICY.

CHAIRPERSON BONNEVILLE: YES.

DR. LEVITT: AND IT'S LIKE, NOT EVEN LIKE, AHEAD OF. IT'S LIKE WAY, WAY, WAY AHEAD OF. AND THAT'S A PROBLEM BECAUSE THERE'S LIKE -- THERE'S CURRENT STATE OPPORTUNITIES. RIGHT? LIKE OUR HOSPITAL, I THINK, HAS, LIKE, 13 CELL AND GENE THERAPIES THAT ARE ONGOING NOW AND WAITING FOR REIMBURSEMENT. AND NOT EVEN KNOWING EXACTLY WHAT THE REIMBURSEMENT IS IS JUST A KILLER. I DON'T KNOW OF A HOSPITAL THAT HAS LARGE AMOUNTS OF GOLD BULLION, CASH ON HAND TO BE ABLE TO -- RIGHT? I MEAN, THAT'S THE PROBLEM. I'LL STOP THERE, BUT IT'S DISHEARTENING.

DR. PATEL: AS CHRISTINA MENTIONED, THERE'S A COUPLE OF -- AND, PAT, AS YOU NOTED -- THERE'S A COUPLE OF POINTS THERE. ONE IS AROUND THE COVERAGE DECISIONS, AND WE CAN TALK A LITTLE BIT MORE ABOUT THE CASE AGREEMENTS HERE BECAUSE OF THE FACT THAT OFTENTIMES THERE'S LIMITED TREATMENT CENTERS. SO PATIENTS ARE TRAVELING TO OTHER STATES, AND THAT CREATES ALL THE AGREEMENTS THAT NEED TO BE MADE BETWEEN THE STATE MEDICAID AGENCIES.

AND, PAT, TO YOUR POINT, WE'VE HEARD

REPEATEDLY FROM VARIOUS PROVIDERS THAT THE DELAYS IN GETTING REIMBURSED ARE REALLY PILING UP ON THEIR BUSINESS LINES IN TERMS OF JUST HOW SLOW SOME PAYERS MAY BE IN MAKING PAYMENT EVEN AFTER THEY'VE AGREED TO DO SO.

CHAIRPERSON BONNEVILLE: KIM.

DR. BARRETT: YEAH. I JUST WANT TO SECOND PAT'S COMMENT ABOUT THE FINANCIAL STRESS THIS PLACES ON PROVIDERS. AND IT WOULD BE SIGNIFICANT NO MATTER WHAT THE TIMING; BUT GIVEN THE CURRENT SET OF STRESSES AND UNCERTAINTIES ON THE SYSTEM FROM FACTORS TOTALLY INDEPENDENT OF ALL THIS, IT'S JUST GOING TO MAKE PEOPLE EVEN MORE CAUTIOUS AND RISK-AVERSE.

AND THEN, SHYAM, JUST FROM AN ACCOUNTABILITY STANDPOINT, COULD YOU TELL US WHY ROCKET WAS GIVEN AN EXTENSION TO PROVIDE THE ACCESS PLAN AND WHEN YOU EXPECT TO RECEIVE THAT?

DR. PATEL: YEAH. SO WITHIN -- AND RAFAEL IS PROBABLY BEST SUITED TO ANSWER THIS QUESTION. BUT WE DO HAVE A MECHANISM WHERE THEY CAN REQUEST AN EXTENSION. AND IN THIS PARTICULAR INSTANCE, BECAUSE THE PRODUCT LAUNCH IS NOT TILL THE END OF THIS YEAR, WE GRANTED THAT EXTENSION.

MR. AGUIRRE-SACASA: THAT'S RIGHT, SHYAM.

KIM, THE IP REGULATIONS ACTUALLY PERMIT UP TO A 30-DAY EXTENSION, AND THE ACCESS PLAN IS DUE MAY 13TH, WHICH IS NEXT WEDNESDAY, IF I'M NOT MISTAKEN.

DR. BARRETT: THANK YOU.

CHAIRPERSON BONNEVILLE: AMMAR.

DR. QADAN: FIRST OF ALL, CONGRATULATIONS. THIS IS REALLY GREAT NEWS FOR PATIENTS.

MY QUESTION IS MORE AROUND THE FACT THAT MANY COUNTRIES OUTSIDE THE U.S., THEY WERE ABLE TO BRING -- LIKE CARVE OUT PATHWAYS AND POLICIES FOR THOSE ULTRA-RARE DRUGS. AND SO THE QUESTION IS SHOULD MAYBE THE BUSINESS MODEL CONSIDER THAT, WHICH IS THE FACT THAT OUTSIDE THE U.S., THERE MIGHT BE A VIABLE WAY BETTER THAN THE U.S.?

CHAIRPERSON BONNEVILLE: SHYAM, DO YOU HAVE ANY COMMENTS ON THAT? ANY OF OUR OTHER GWG -- OR AAWG MEMBERS?

DR. PATEL: AND THIS IS ONE OF THE AREAS THAT CIRM IS INVESTIGATING MORE DEEPLY IS AROUND THE -- THERE'S SO MUCH, SO MANY OTHER FINANCIAL INSTRUMENTS AT PLAY HERE. THERE'S STOP LOSS, THERE'S CARVE-OUTS, AND ALL THAT THAT ARE BEING DONE. THERE'S ALSO NEWER COMPANIES EMERGING THAT ARE DOING THIS. AND WE'RE TRYING TO UNDERSTAND REALLY HOW THOSE ARE BEING LEVERAGED FOR CELL AND

GENE THERAPIES, WHAT'S ACTUALLY EFFECTIVE, WHAT'S ACTUALLY BEING USED, AND HOW DOES THAT IMPACT BOTH THE PROVIDER SIDE AS WELL AS THE PAYER SIDE.

FOR EXAMPLE, MANY COMPANIES NOW HAVE BEEN LEVERAGING SPECIALTY PHARMA SOLUTIONS TO BE THE INTERMEDIARY BETWEEN THE MANUFACTURER AND THE PROVIDER. AND SO IN THAT INSTANCE, WE DON'T KNOW HOW EFFECTIVE THAT HAS BEEN ACROSS SOME OF THE MORE EXPENSIVE CELL AND GENE THERAPIES AS WELL AS SOME OF THE OTHER FINANCIAL INSTRUMENTS. THAT'S PART OF WHAT WE WANT TO ADDRESS GOING FORWARD IS UNDERSTANDING PRACTICALLY HOW THOSE ARE ACTUALLY BEING IMPLEMENTED AND HOW THEY'RE BENEFITING THE HEALTHCARE SYSTEM.

CHAIRPERSON BONNEVILLE: DARIUS.

DR. LAKDAWALLA: I THINK AMMAR MAKES A GREAT POINT. AND JUST ONE PRACTICAL NOTE I'LL ADD TO THAT IS, TO THE EXTENT THAT THAT IS GOING TO BE PURSUED, THEN COMPANIES HAVE TO HAVE A VERY SPECIFIC AND APPROPRIATE HEALTH TECHNOLOGY ASSESSMENT APPLICATION PLAN.

AND I MEAN THE GOOD NEWS IS THAT THERE'S A LOT -- THERE ARE A LOT OF EXCEPTIONS MADE FOR RARE DISEASES INCREASINGLY AROUND THE WORLD. MAYBE THAT WILL EXPAND WITH MOST FAVORED NATION PRESSURE, I

DON'T KNOW; BUT WHATEVER THE CASE IS, IT'S IMPORTANT FOR PEOPLE TO BE READY FOR THAT KIND OF OPPORTUNITY BECAUSE IT'S PROBABLY WORTHWHILE.

CHAIRPERSON BONNEVILLE: THANK YOU. IF THERE ARE NO OTHER COMMENTS OR QUESTIONS FOR THIS AGENDA ITEM, SHYAM?

DR. PATEL: THANK YOU. SO A COUPLE OPERATIONAL UPDATES ON OUR SIDE. THIS NEXT ONE I'M GOING TO MAKE QUICK, AND THEN I'LL TURN IT OVER TO GEOFF TO TALK ABOUT THE CCCE.

SO AS YOU REMEMBER, LAST YEAR WE WORKED WITH THE WORKING GROUP HERE TO DEVELOP A SET OF REQUIREMENTS AND GUIDANCES FOR OUR PRECLINICAL AND CLINICAL STAGE AWARDEES TO INCORPORATE ACCESS AND AFFORDABILITY PLANNING STRATEGIES INTO THEIR CIRM AWARD AND FOR US TO BE ABLE TO TRACK STAGE-APPROPRIATE PLANNING AS THEY PROGRESS FROM PRECLINICAL TO EARLY CLINICAL TO LATE CLINICAL.

AND SO IN THAT RESPECT, WE HAVE UPDATED SOME OF THAT BASED ON THE LAST FEW MONTHS OF INTERACTIONS WITH THE BOARD. SO OVER THE LAST FEW MONTHS WITH THE BOARD, THE CIRM TEAMS ARE WORKING CLOSELY TO RE-EVALUATE HOW WE SELECT, REVIEW, AND FUND APPLICATIONS FOR OUR CORE FUNDING PROGRAMS. THIS IS DISCOVERY, PRECLINICAL DEVELOPMENT, AND

CLIN2. AND PAT HAS BEEN A VERY STRONG VOICE IN THAT AS WELL AS REALLY SUPPORTING US THROUGH THAT PROCESS.

SO AS PART OF THAT, THERE WAS ALSO FEEDBACK ON THE ACCESS AND AFFORDABILITY PLANNING REQUIREMENTS. IN PARTICULAR, THE BOARD AGREED THAT ACCESS STRATEGY IS REALLY IMPORTANT AND IT SHOULD BE A CONSIDERATION IN CERTAIN FUNDING DECISIONS AS WELL AS SOMETHING THAT IS TRACKED OVER THE COURSE OF THE AWARD.

HOWEVER, THEY DID NOT FEEL THAT AFFORDABILITY, GIVEN THE COMPLEXITY OF AFFORDABILITY IN THE U.S. HEALTHCARE SYSTEM, THAT THAT SHOULD BE SOMETHING THAT'S FACTORED INTO THE CIRM FUNDING DECISION ON AN INDIVIDUAL PROJECT LEVEL. AND THEY ALSO WANTED TO MAKE SURE THAT WE PROVIDED CLEAR GUIDANCE TO ALL APPLICANTS AND REVIEWERS ABOUT WHAT WE MEAN BY HAVING A CLEAR PATIENT ACCESS STRATEGY.

AND FINALLY, THEY WANTED TO MAKE SURE THAT THE PATIENT VOICE AND EXPERT INPUT ON THIS ARE PRESERVED IN THE EVALUATION PROCESS. AND SO COMING OUT OF ALL OF THAT, TAKING INTO ACCOUNT BOARD'S APPROVALS AND RECOMMENDATIONS, WE HAVE UPDATED HOW WE EVALUATE ACCESS STRATEGIES IN OUR PRECLINICAL AND CLINICAL PROGRAMS.

SO FIRST OF ALL, WE REFRAMED IT AS A PATIENT ACCESS STRATEGY TO FOCUS MORE BROADLY ON ACCESS CONSIDERATIONS THAT ARE WITHIN THE CONTROL OF THE SPONSOR.

WE'VE ALSO MADE SURE THAT THE ACCESS PLANS ARE STAGE APPROPRIATE ACROSS THOSE DIFFERENT STAGES OF DEVELOPMENT AND HAVE IMPROVED INSTRUCTIONS, REVIEW CRITERIA, AND TRAINING TO ENSURE THAT THIS IS ALL BEING FAIRLY ADJUDICATED DURING THE APPLICATION SELECTION, RECOMMENDATION, AND APPROVAL PROCESS.

AND OVERALL, WE'VE ACTUALLY LEANED MORE HEAVILY INTO PATIENT ACCESS STRATEGY BEING A KEY COMPONENT OF THE CLIN2 AND PDEV APPLICATIONS IN THE SENSE THAT THIS IS NOW BEING INCORPORATED INTO THE GWG REVIEW AS A WHOLE.

AND FINALLY, THE PATIENT ADVOCATE BOARD MEMBERS WHO PARTICIPATE IN THE GRANTS WORKING GROUP REVIEW WILL HAVE A MORE DEFINED SCORING ROLE. AND SO THEIR VOICE WILL BE MORE CLEAR AND WILL ALSO REFLECT IT IN THEIR SPECIFIC SCORING AS TO HOW THEY FELT ABOUT THE APPLICATION, ITS PATIENT ACCESS STRATEGY, HOW IT'S IMPACTING THIS CALIFORNIA POPULATION, AND HOW IT'S POSITIONED TO MOVE FORWARD.

CHAIRPERSON BONNEVILLE: THANK YOU, SHYAM. ARE THERE ANY COMMENTS FROM ANY OF THE BOARD MEMBERS

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ABOUT THIS? I MEAN ANYONE CAN COMMENT, OBVIOUSLY, BUT BOARD MEMBERS WHO WENT THROUGH THIS PROCESS.

PAT?

DR. LEVITT: WHAT DO YOU WANT ME TO SAY? I MEAN WE HAD LOTS OF ROBUST CONVERSATION ABOUT THIS. I THINK THE WAY IT'S STRUCTURED NOW, I THINK, MAKES SENSE FROM AN INVESTIGATOR PERSPECTIVE IN TERMS OF WHAT THEY SHOULD BE PAYING ATTENTION TO AND WHAT THEY NEED TO BE DEALING WITH. AND I THINK THE ROLE OF THE PATIENT ADVOCATES, WHICH I HEARD ABOUT MORE YESTERDAY AT ANOTHER CIRM MEETING, I THINK IT'S GREAT. SO I THINK IT'S BEEN INCORPORATED AND INTEGRATED REALLY WELL. AND I THINK THE AFFORDABILITY COMPONENT, WHICH WE'VE HEARD AT THIS MEETING, IT'S JUST BEYOND WHAT AN INVESTIGATOR TEAM, RIGHT, APPLYING FOR A GRANT IS GOING TO BE ABLE TO DEAL WITH.

SO WHILE THEY CAN CERTAINLY ACKNOWLEDGE THESE CHALLENGES, I THINK EXPECTING THEM TO COME UP WITH A PLAN. SO, SHYAM AND TEAM, I THINK YOU ALL CAME UP WITH GREAT FRAMING FOR THIS. LOOKS GOOD.

CHAIRPERSON BONNEVILLE: THANK YOU.

IF THERE ARE NO OTHER COMMENTS, I THINK WE ARE AT OUR LAST AGENDA ITEM, AND THAT'S GEOFF LOMAX.

DR. LOMAX: GOOD MORNING, EVERYONE, AND

THANK YOU. COULD WE GO TO THE FIRST SLIDE THERE. THANKS VERY MUCH.

SO THE AIM OF THIS PRESENTATION IS TO UPDATE YOU ALL ON THE COMMUNITY CARE CENTERS OF EXCELLENCE PROGRAM. I THINK, AS MOST OF YOU ARE AWARE, THIS IS A PROGRAM THAT IS CALLED OUT IN PROPOSITION 14 WITH THE AIM OF EXPANDING CIRM'S CLINICAL INFRASTRUCTURE, SPECIFICALLY SITES THAT CAN PERFORM CELL AND GENE THERAPY CLINICAL TRIALS AND THE DELIVERY OF APPROVED PRODUCTS TO EXPAND THOSE DELIVERY PLATFORMS TO AREAS IN CALIFORNIA THAT HAVE HISTORICALLY BEEN UNDERSERVED OR CERTAINLY PATIENTS WOULD HAVE TO TRAVEL CONSIDERABLE DISTANCE IF THEY WERE TO ACCESS THE ACADEMIC MEDICAL CENTERS WITHIN OUR EXISTING ALPHA CLINICS NETWORK.

SO YOU ALL WERE VERY INFORMATIVE FROM THE GET-GO, BOTH IN TERMS OF OUR EARLY NEEDS ASSESSMENT THROUGH TO THE CONCEPT PLAN. LAST FALL WE WERE ABLE TO BRING A SET OF RECOMMENDATIONS TO THE BOARD WHERE THEY APPROVED THREE AWARDS, AND THESE AWARDS WERE LAUNCHED ON MARCH 1ST.

WHEN WE SAY LAUNCH, THAT MEANS WE'VE SIGNED THE CONTRACTS WITH THE AWARDEES, AGREED TO TERMS AND CONDITIONS, MILESTONES, ALL THE THINGS THAT ARE BEHIND A SUCCESSFUL CIRM AWARD.

SORRY. COULD YOU JUST STAY ON THAT SLIDE FOR ONE MOMENT? YEAH. THANK YOU. EACH OF THE AWARDS IS 9 MILLION. THEY ARE FIVE-YEAR AWARDS. AND AS PART OF -- IMMEDIATELY AFTER KICKOFF, THE FIRST ORDER OF BUSINESS, IF YOU WILL, WAS TO HAVE AN ORIENTATION SESSION WITH THE ALPHA CLINICS. AS PART OF THE AWARD, THE AWARD REQUIRES EXTENSIVE COLLABORATION AND COORDINATION WITH OUR EXISTING CLINICAL TRIAL NETWORK. THOSE DISCUSSIONS WERE, I WOULD CHARACTERIZE AS -- WHAT WAS REALLY HEARTENING TO SEE IS THEY WERE VERY BI-DIRECTIONAL WHERE THE COMMUNITY CARE CENTERS HAD ALREADY HAD ASSETS THEY WERE BRINGING TO THE TABLE THAT THE ALPHA CLINICS WERE INTERESTED IN. AND THE ALPHA CLINICS, IN RETURN, WERE WILLING AND ABLE PARTNERS IN AREAS WHERE SOME OF THE SITES WERE LOOKING FOR SUPPORT.

MOST NOTABLY, I THINK WHERE THE ALPHA CLINICS CAN BE AN IMMEDIATE ASSIST TO THE LAUNCHING PROGRAMS ARE IN SOME OF THE AREAS AROUND TRAINING AND DELIVERY OF PRODUCTS. THERE WAS DEFINITELY SOME REQUESTS IN THAT AREA AROUND TRAINING, BOTH ON THE DELIVERY SIDE AND EVEN THE ADMINISTRATION AND HOW TO MOVE THESE PRODUCTS THROUGH THEIR SERVICE LINES.

SO, AGAIN, GOOD DISCUSSIONS. PEOPLE ARE GETTING CONNECTED. AND THOSE PEER-TO-PEER

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CONNECTIONS ARE STARTING.

THE CIRM TEAM WILL BE VISITING EACH OF THE SITES OVER THE NEXT ABOUT SIX WEEKS WITH THE AIM OF, AGAIN, GETTING INTO A BIT MORE DETAIL IN TERMS OF NEEDS, HOW WE CAN HELP, AND JUST TO ADDRESS BREAD-AND-BUTTER QUESTIONS ABOUT THE AWARDS THEMSELVES, THE ADMINISTRATION, AND THE OVERALL PROGRAM.

SO WITH THAT, COULD WE GO TO THE NEXT SLIDE, PLEASE. JUST GIVE YOU A LITTLE BIT OF COLOR ON THE THREE AWARDS WE HAVE SO FAR. THE SITE, THE LUNDQUIST SITE, IS IN TORRANCE, CALIFORNIA. AND THAT SITE IS REALLY FOCUSED ON -- THIS IS OUR FIRST REAL BIG PUSH INTO ENGAGING PATIENTS THAT WOULD BE IN THE COUNTY HEALTH SYSTEMS. SO THESE ARE HISTORICALLY UNDERSERVED PATIENTS ACROSS THE HEALTHCARE SPECTRUM, INCLUDING, CERTAINLY, CLINICAL TRIALS AND REGENERATIVE MEDICINE THERAPIES AS WELL. AND SO THAT SITE REALLY PROPOSES VERY MUCH A DEEP DIVE INTO POPULATIONS IN SOUTH CENTRAL LOS ANGELES THAT ARE PRIMARILY SERVED THROUGH THE COUNTY HOSPITAL SYSTEM. AND ALREADY THERE'S BEEN A LOT OF ENGAGEMENT WITH BOTH UC IRVINE AND UCLA'S ALPHA CLINIC AND CEDARS. SO SORT OF ALPHA CLINICS THAT SORT OF TOUCH ON THOSE BOUNDARIES ALREADY OFFERING

SUPPORT WHERE THAT SITE HAS BEEN INTERESTED IN, AGAIN, GETTING TECHNICAL ASSISTANCE, TRAINING, AND SUPPORT.

THE INLAND EMPIRE, WHICH IS LOMA LINDA, THAT REALLY GIVES US NOW A MUCH MORE EXPANSIVE FOOTPRINT INTO RIVERSIDE COUNTY, THE DESERT REGION. AND LOMA LINDA, VERY INTERESTING, IS VERY KEEN TO LEAN INTO AREAS OF THE NEUROLOGICAL SPACE. THAT IS SOMETHING THAT THEY'RE DEVELOPING AT THEIR CENTER AND HAVE BECOME VERY INTERESTED IN THE PROGRAMS THAT ALIGN NICELY WITH OUR EFFORTS TO SUPPORT DISEASES OF THE BRAIN AND NERVOUS SYSTEM. SO LOMA LINDA SHOULD BE A BIG ASSET IN TERMS OF THOSE PROGRAMS.

AND THEN COMMUNITY HEALTH SYSTEMS IN FRESNO, THEY, INITIALLY IN THEIR APPLICATION AT THE TIME, FOCUSED QUITE A BIT ON CAR-T AND CANCER ONCOLOGY, BUT ARE ALREADY VERY MUCH, AS IS A TREND IN THE FIELD, REALLY LOOKING NOW AT CAR-T AS A PLATFORM THAT COULD TREAT ACROSS A NUMBER OF DISEASE AREAS, INCLUDING, BUT NOT LIMITED TO, ONCOLOGY AND HOW TO MAKE SURE THAT THEY'VE GOT A DELIVERY SYSTEM THAT IS ACCREDITED AND ABLE TO MANAGE CONDITIONS, AGAIN, THAT MAY BE AMENABLE TO CAR-T THERAPY ACROSS THE SPECTRUM. SO VERY EXCITING PROGRAM. AND, AGAIN, WE'LL BE VISITING THESE SITES AND LEARNING

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MORE OVER THE NEXT FEW WEEKS.

NEXT SLIDE, PLEASE. SO ONE THING THAT DID COME UP WHEN WE BROUGHT THESE PROGRAMS FORWARD TO THE BOARD WAS A CONCERN, IF YOU LOOK AT THE GRAPH OR THE PICTURE TO THE RIGHT HERE, IS THAT OUR CLINICAL INFRASTRUCTURE, WHILE EXPANDING TO AREAS THAT, AGAIN, WERE HISTORICALLY UNDERSERVED AND WE WERE ACHIEVING THE AIMS OF THE PROGRAM, THERE WAS STILL NO AWARD THAT WAS RECOMMENDED FOR FUNDING THAT WOULD COVER THE NORTHERN THIRD OF CALIFORNIA. THIS REPRESENTS APPROXIMATELY 3 MILLION RESIDENTS. IT'S GEOGRAPHICALLY ONE-THIRD OF THE AREA OF THE STATE. AND THE BOARD ASKED US TO GO BACK AND EXPLORE OPPORTUNITIES TO IDENTIFY OTHER SITES THAT WE COULD BRING BACK IN, IN PART BECAUSE OF THE ORIGINAL CONCEPT PLAN, THE BUDGET DID ALLOW FOR THE FUNDING, WOULD ALLOW FOR UP TO FOUR AWARDS. AND AS I JUST MENTIONED, THREE WERE RECOMMENDED FOR FUNDING AND SUBSEQUENTLY APPROVED BY THE BOARD.

SO THERE WAS A BUDGET REMAINING AS WELL. SO IN MARCH THE ICOC VOTED TO REOPEN THE PROGRAM, ALLOWING FOR THAT REMAINING BUDGET TO BE DEPLOYED IF WE WERE TO HAVE A SUCCESSFUL APPLICANT COME THROUGH THE PROCESS. BUT SPECIFICALLY WHAT'S UNIQUE TO THAT REOPENING IS THERE IS A PROVISION THAT WILL LIMIT

THE APPLICANTS TO CENTERS THAT ARE GEOGRAPHICALLY LOCATED WITHIN THE MAP, WHICH I'M SHOWING YOU, WITHIN THIS AREA HERE.

SO THE APPLICATION IS CURRENTLY OPEN. IT WILL BE OPEN TILL THE BEGINNING OF JUNE. AND WE'VE HAD SOME PRODUCTIVE INTERACTIONS WITH POTENTIAL APPLICANTS. SO WE LOOK FORWARD TO RECEIVING ROBUST APPLICATIONS WITH THE HOPE THAT THEY WILL BE REVIEWED FAVORABLY ONCE THEY ARE SUBMITTED.

AND I THINK THAT COVERS IT. IS THERE ANYTHING ELSE IN THE DECK HERE?

MR. MALIK: THAT'S IT.

DR. LEVINE: HEY. HEY, GEOFF, CAN I ASK A COUPLE -- I GUESS I SHOULD RAISE MY HAND.

CHAIRPERSON BONNEVILLE: HARLAN, GO AHEAD.

DR. LEVINE: I DON'T SEE ANYONE AHEAD OF ME HERE.

CHAIRPERSON BONNEVILLE: NO. NO. GO AHEAD. I LIKE THAT.

DR. LEVINE: YEAH.

CHAIRPERSON BONNEVILLE: OLD SCHOOL HAND RAISE.

DR. LEVINE: GEOFF, I KNOW WE HAVE RIGOROUS CRITERIA FOR INCLUSION OR GETTING GRANTED. WHAT DO WE HAVE SET UP TO MONITOR? AND THEN DO WE

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HAVE KPI'S TO KNOW THAT WE'RE BEING SUCCESSFUL?

DR. LOMAX: WE DO. WE'VE ACTUALLY -- THAT WAS A VERY EXTENSIVE EFFORT EARLY ON. IN THE CONTRACTING PROCESS, WE HAD A LOT OF BACK AND FORTH WITH THE AWARDEES. THEY'VE AGREED TO WHAT I WOULD CHARACTERIZE AS A VERY ROBUST SET OF KPI'S. I DON'T HAVE THEM ON THE TIP OF MY TONGUE, BUT --

DR. LEVINE: YEAH. BUT THEY'RE ESTABLISHED. OKAY.

DR. LOMAX: THEY'RE ESTABLISHED. AND I THINK, TO THE EXTENT THEY'RE DIFFERENT THAN THE ALPHA CLINICS, BECAUSE I HOPE THIS GIVES YOU A LITTLE MORE DEPTH IN TERMS OF WHERE WE'RE GOING WITH THIS, THIS GOES BACK TO, JUST IF YOU MAY RECALL, VERY EARLY DISCUSSIONS ABOUT WHAT MEASURES OF SUCCESS WOULD BE FOR THIS PROGRAM. AND IT WAS BRINGING VISIBILITY, CERTAINLY VISIBILITY, AND OPTIMALLY REFERRALS OF PATIENTS WHO OTHERWISE JUST HISTORICALLY HAVE NOT HAD VISIBILITY OR ACCESS TO CLINICAL TRIALS, THAT MOVING PATIENTS FROM, SAY, THE PRIMARY CARE OR SPECIALTY CARE PHYSICIAN LEVEL TO AT LEAST HAVING AN OPPORTUNITY TO KNOW THAT THERE IS A CLINICAL TRIAL THAT MAY BE APPLICABLE OR IS APPLICABLE TO THEIR CONDITION AND HAVING THEM HAVE THAT OPPORTUNITY TO PARTICIPATE.

AND SO THE KPI'S, PARTICULARLY FOR THE COMMUNITY CARE CENTER PROGRAMS, REALLY DO TRY TO GET AT THAT CHAIN OF THAT PATIENT JOURNEY FROM EARLY AWARENESS, ENGAGEMENT, THROUGH MULTIPLE -- ENGAGEMENT THROUGH MULTIPLE AREAS, THROUGH BOTH CAREGIVER, COMMUNITY-BASED ORGANIZATIONS, PATIENT ADVOCACY ORGANIZATIONS, THAT INITIAL ENGAGEMENT TO CLINICAL TRIAL OPPORTUNITY, AND CAPTURING METRICS ALONG THAT CHAIN TO SEE IF WE CAN MOVE THE NEEDLE IN TERMS OF ACCESS TO POPULATIONS THAT HAVE HISTORICALLY BEEN UNDERREPRESENTED IN CLINICAL TRIALS.

DR. LEVINE: THANK YOU.

DR. LOMAX: AND I MIGHT JUST ADD THAT WAS SOMETHING THE SCIENCE SUBCOMMITTEE WAS VERY ADAMANT ABOUT IN TERMS OF PROGRAM DESIGN. SO I WANT TO ACKNOWLEDGE THE SCIENCE SUBCOMMITTEE'S ROLE IN PUSHING US TO MAKE SURE WE CAPTURE THAT ASPECT OF THE PATIENT JOURNEY.

CHAIRPERSON BONNEVILLE: ARE THERE ANY OTHER QUESTIONS FROM ANY OF THE MEMBERS OF THE WORKING GROUP?

I DO WANT TO ADD, THIS PROGRAM, WHEN WE TALK ABOUT IT OUTSIDE OF CIRM AND OUTSIDE OF THE USUAL FOLKS THAT WE INTERACT WITH AND GO BEYOND TO

NEW STAKEHOLDERS AND PEOPLE WHO ARE INTERESTED IN CIRM, THIS IS UNIVERSALLY A PROGRAM THAT EVERYONE FINDS INNOVATIVE AND THRILLING AND EXCITED THAT WE'VE UNDERTAKEN THIS SORT OF PROGRAM.

SO I WANT TO THANK THE TEAM FOR REALLY WORKING HARD TO MAKE IT WHAT IT IS AND LOOK FORWARD TO SEEING HOW IT WORKS.

I WOULD BE REMISS IF I DID NOT ASK FOR ANY PUBLIC COMMENT. CAMERON, DO YOU KNOW IF THERE'S ANY PUBLIC COMMENT?

MR. MALIK: THERE DOES NOT APPEAR TO BE PUBLIC COMMENT IN THE YOUTUBE CHAT. BUT FOR THOSE WHO WANT TO MAKE A PUBLIC COMMENT, PLEASE PRESS STAR AND 9 TO RAISE YOUR HAND IN ZOOM, AND THEN STAR 6 TO UNMUTE WHEN WE CALL ON YOU. SO STAR 9 IF YOU'D LIKE TO MAKE PUBLIC COMMENT. THERE DOES NOT APPEAR TO BE ANY PUBLIC COMMENT.

CHAIRPERSON BONNEVILLE: GREAT. THANK YOU. I WANT TO THANK EVERYONE FOR TAKING TIME, AND WE'VE GONE A FEW MINUTES OVER SCHEDULE, BUT I REALLY APPRECIATE ALL THE WORK THAT YOU ALL PUT IN AND YOUR SERVICE TO CIRM. AND WE WILL SEE YOU AGAIN SOON. THANKS SO MUCH. HAVE A GREAT WEEKEND.

(THE MEETING WAS THEN CONCLUDED.)

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 ACCESS 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 8, 2026, 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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