BEFORE THE
ACCESSIBILITY AND AFFORDABILITY WORKING GROUP OF THE
INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT

REGULAR MEETING

LOCATION: VIA ZOOM

DATE: MARCH 14, 2023
8 A.M.

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

FILE NO.: 2023-11

I N D E X

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TUESDAY, MARCH 14, 2023; 1 P.M.

VICE CHAIR BONNEVILLE: GOOD AFTERNOON, EVERYONE. THANK YOU FOR JOINING US TODAY. MARIANNE, CAN YOU PLEASE CALL THE ROLL.

MS. DEQUINA-VILLABLANCA: DAN BERNAL.

MR. BERNAL: PRESENT.

MS. DEQUINA-VILLABLANCA: MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MS. DEQUINA-VILLABLANCA: ANN BOYNTON.

MS. BOYNTON: PRESENT.

MS. DEQUINA-VILLABLANCA: JAMES DEBENEDETTI. DANA DORNSIFE. DAVID GOLDMAN. TED GOLDSTEIN.

MR. GOLDSTEIN: HERE.

MS. DEQUINA-VILLABLANCA: DAVID HIGGINS. HARLAN LEVINE.

DR. LEVINE: HERE.

MS. DEQUINA-VILLABLANCA: PAT LEVITT.

DR. LEVITT: HERE.

MS. DEQUINA-VILLABLANCA: ADRIANA PADILLA.

DR. PADILLA: HERE.

MS. DEQUINA-VILLABLANCA: AMMAR QADAN.

DR. QADAN: PRESENT.

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MS. DEQUINA-VILLABLANCA: AL ROWLETT.

MR. ROWLETT: HERE.

MS. DEQUINA-VILLABLANCA: DAVID SERRANO-SEWELL. MAHESWARI SENTHIL. ADRIENNE SHAPIRO.

MS. SHAPIRO: PRESENT.

MS. DEQUINA-VILLABLANCA: JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MS. DEQUINA-VILLABLANCA: OKAY. WE'VE GOT IT.

VICE CHAIR BONNEVILLE: THANK YOU, MARIANNE.

SO TODAY THE TEAM IS PRESENTING A USE CASE FOR THE ACCESS AND AFFORDABILITY ROADMAP RARE DISEASE. AS YOU ALL KNOW, 50 PERCENT OF OUR PORTFOLIO IS IN DISEASE INDICATIONS. HOWEVER, IT IS THE TEAM'S FEELING THAT THE METHODS AND STRATEGIES PRESENTED WILL BE BROADLY APPLICABLE ACROSS OUR PORTFOLIO PROGRAMS.

AS WE GET CLOSER TO JUNE, IT IS VITAL THAT THIS GROUP WEIGH IN ON THE STRATEGIC VISION THAT IS PRESENTED BY THE TEAM, TO THE ACTIVITIES AND PROGRAMS ENVISIONED SEEM REASONABLE AND EFFECTIVE, AND DO THEY FURTHER THE GOALS OF THE ORGANIZATION.

AND SO WITH THAT, I'LL TURN IT OVER TO

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SEAN TO TAKE US THROUGH THIS.

DR. TURBEVILLE: WELL, THANK YOU. I'M GOING TO GO AHEAD AND SHARE MY SLIDES. MAKE SURE WE GET A THUMBS UP YOU CAN SEE THESE.

WONDERFUL. VICE CHAIRMAN, NEWLY ELECTED VICE CHAIRMAN, I DON'T KNOW HOW MANY TIMES I HAVE TO SAY THAT, BUT THIS WILL BE THE LAST TIME, OR NOT EVEN CLOSE. IT'S COMING. ALL RIGHT. THANK YOU FOR THE CORRECTION.

AAWG, THANK YOU FOR ATTENDING THIS MEETING. THIS IS ACTUALLY A PRETTY ENTHUSIASTIC PRESENTATION FROM MY PERSPECTIVE. AS BONNEVILLE TEED UP, WE ARE GETTING READY TO PRESENT A ROADMAP FOR THE ACCESS AND AFFORDABILITY. AND THAT ROADMAP IS, WITHIN A COUPLE MORE PRESENTATIONS, GOING TO BE FINE-TUNED AND, AS WE DISCUSSED EARLIER, BE PRESENTED TO THIS TEAM, THIS CROSS-FUNCTIONAL TEAM, THAT'S GOT A LOT OF EXPERTISE THAT CAN GIVE US GUIDANCE ON FINE-TUNING THAT FOR THE ICOC PRESENTATION LATER THIS SUMMER.

SO AS BONNEVILLE MENTIONED, WE'RE GOING TO TALK ABOUT RARE DISEASE AS THE USE CASE FOR ACCESS AND AFFORDABILITY. AND A COUPLE OF SCENARIOS THAT I REALLY WANT THE TEAM TO CONSIDER FROM POTENTIALLY A FUNDING OPPORTUNITY DOWN THE ROAD, WHETHER THAT'S IN

MEDICAL AFFAIRS OR EVEN ON THE CLINICAL SIDE.

SO JUST AS A REMINDER, THESE ARE THE AAWG-APPROVED CATEGORIES WE ARE FRAMING FOR OUR DISCUSSION AROUND ACCESS AND AFFORDABILITY. WE ARE, OF COURSE, ALIGNING WITH OUR FIVE-YEAR STRATEGIC PLAN. WE ARE PROVIDING DIFFERENT COMPONENTS LISTED HERE FOR CONSIDERATION. WE'VE TOUCHED ON SEVERAL OF THESE COMPONENTS IN OUR PREVIOUS PRESENTATIONS.

TODAY I WANT TO FOCUS SPECIFICALLY ON RARE DISEASE AND, MORE IMPORTANTLY, THE AREA THAT I FOCUSED HERE UNDER BUILD CLINICAL RESOURCES. AND THAT IS THE POST-MARKETING SURVEILLANCE SCENARIO OR AREA. AND I DON'T KNOW IF MANY OF YOU HAVE BEEN FOLLOWING THE LITERATURE, BUT THIS IS AN AREA THAT'S ACCUMULATED A NUMBER OF CURRENT PUBLICATIONS IN THE LAST TWO WEEKS FOCUSING ON THE IMPORTANCE OF POST-MARKETING SURVEILLANCE FOR CELL AND GENE THERAPY TRIALS. THERE ARE MANY WHITE PAPERS MAYBE IN THE LAST TWO WEEKS, INCLUDING A PUBLICATION IN *NEW ENGLAND JOURNAL OF MEDICINE* FROM A FORMER FDA COMMISSIONER ABOUT THE IMPORTANCE OF POST-MARKETING SURVEILLANCE, AGAIN, FOR CELL AND GENE THERAPIES.

SO IT'S SORT OF REASSURING FROM A MEDICAL AFFAIRS STANDPOINT BECAUSE WE'RE TRYING TO PREDICT TO SOME EXTENT WHAT THIS ROADMAP IS GOING TO LOOK

LIKE IN THE THREE- TO FIVE-YEAR PROJECTIONS. AND AT THE SAME TIME, I THINK J.T. MENTIONED DURING OUR COMMUNITY CARE CENTERS OF EXCELLENCE THAT ALL OF THIS DATA IS HAPPENING REAL-TIME. THE PAYER SIDE IS HAPPENING IN REAL TIME, ACCESS AND AFFORDABILITY SORT OF HAPPENING REAL-TIME. SO THERE'S NO REAL BENCHMARK THAT WE CAN LOOK AT FOR TRYING TO PREDICT WHAT WE CAN PUT IN PLAY FROM AN ACCESS AND AFFORDABILITY STANDPOINT MOVING FORWARD IN THE NEXT THREE TO FIVE YEARS.

SO TODAY'S FOCUS, AND I KNOW ALL OF YOU ARE TERRIBLY BUSY, TRY TO KEEP THIS CONCISE, IS, AS BONNEVILLE MENTIONED, USE RARE DISEASE AS A USE CASE FOR DISCUSSION OF THE ROADMAP TO ACCESS AND AFFORDABILITY. AND AS BONNEVILLE MENTIONED, WHY IS THAT THE CASE? WELL, APPROXIMATELY HALF, ACTUALLY EXACTLY 53 PERCENT OF CIRM'S PORTFOLIO IS IN THE RARE DISEASE SPACE AND ADVANCING TO LATER DEVELOPMENT CLINICAL STAGES IN THE CLINIC.

SO STARTING TO TAKE AWARE OF HOW THESE ARE PROGRESSING THROUGH THE CLINIC. MANY OF YOU ARE FAMILIAR WITH THE RARE DISEASE LANDSCAPE. THERE ARE APPROXIMATELY 7,000 RARE DISEASES IN THE UNITED STATES AFFECTING AN ESTIMATED 25 TO 30 MILLION PEOPLE OF WHICH 50 PERCENT ARE IN THE PEDE SPACE OR

CHILDREN. SO CUMULATIVELY A BIG IMPACT FACTOR FOR RARE DISEASES.

SO TO GIVE YOU AN IDEA OF THE THERAPEUTIC AREAS THAT CIRM HAS INVESTED IN, THIS IS A SLIDE THAT SHOWS THE ACTIVE CLINICAL TRIAL PORTFOLIO IN THE RARE DISEASE SPACE. SO WE CURRENTLY HAVE 26 ACTIVE CLINICAL TRIALS. OF THOSE 26, 46 PERCENT ARE IN THE HEMATOLOGICAL SPACE. SICKLE CELL IS SORT OF ON THE BORDERLINE OF, QUITE FRANKLY, A RARE DISEASE THAT HAS A HIGH INCIDENCE AND PREVALENCE IN THE UNITED STATES AND EVEN GLOBALLY, BUT THERE ARE A NUMBER OF OTHER HEMATOLOGICAL CONDITIONS HERE THAT ARE IN OUR PORTFOLIO, MANY OF WHICH HAVE ABSOLUTELY NO ALTERNATIVE TREATMENT OPTIONS.

SO YOU THINK ABOUT THE INVESTMENTS THAT CIRM MADE IN MAKING AN IMPACT OR POTENTIALLY MAKING AN IMPACT IN THIS RARE DISEASE SPACE IS ACTUALLY QUITE IMPRESSIVE.

31 PERCENT OF THE PORTFOLIO IS IN THE NEUROLOGICAL SPACE. WE THINK ABOUT AML, SPINA BIFIDA, ONCOLOGY, AND THE BRAIN, BRAIN CANCERS, AND, JUST TO DATE MYSELF, MUCOPOLYSACCHARIDOSIS 1, WHICH IS HURLER SYNDROME. I ACTUALLY WAS INVOLVED WITH THE FIRST ENZYME REPLACEMENT THERAPY FOR THE LAUNCH OF A THERAPY FOR THAT MPS DISEASE, WHICH IS A

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DIFFICULT, GUT-WRENCHING DISEASE THAT HAS MADE A LOT OF HEADWAY FROM A SCIENTIFIC AND THERAPEUTIC STANDPOINT.

8 PERCENT ARE IN THE BLOOD CANCERS, YOU THINK ABOUT AML TRANSPLANTATION; 4 PERCENT IN THE MUSCULOSKELETAL AREA; 8 PERCENT IN OCULAR; AND ALSO 4 PERCENT IN THE RENAL CYSTINOSIS. THIS SLIDE WAS PRESENTED TO US OR PROVIDED BY ABLA AND HER TEAM. I WANT TO THANK HER TEAM FOR PUTTING THIS TOGETHER. WHEN YOU THINK ABOUT RARE DISEASES, THIS AFFECTS CHILDREN AND ADULTS. WE ARE UTILIZING STATE-OF-THE-ART TECHNOLOGIES. AND, AGAIN, WHEN WE START THINKING ABOUT ADVANCING TOWARDS REGISTRATION, WE HAVE 20 TRIALS RIGHT NOW THAT ARE IN PHASE 1, FIVE TRIALS THAT ARE IN PHASE 1 AND 2, AND ONE THAT'S IN PHASE 2. AND, AGAIN, 26 ACTIVE CLINICAL TRIALS IN THE RARE DISEASE SPACE, AND THIS IS AS OF 2/27/2023.

SO THIS IS A TABLE LISTING THE ACTIVE RARE DISEASE GRANTS WITH ACCELERATED DESIGNATION. SO THIS IS IMPORTANT. ONE, WE LIST, JUST WALKING YOU THROUGH THIS TABLE, THE CLINICAL PHASE, WHICH I MENTIONED EARLIER WITH ACCELERATE DESIGNATIONS, WE HAVE A NUMBER OF TRIALS, AGAIN, PHASE 1, 1 AND 2 COMBINED. THE DISEASE AREA WITH RESPECT TO MOSTLY

IN THE HEMATOLOGICAL AREA, WE HAVE ONE IN LEUCOCYTE ADHESION DEFICIENCY 1 DISORDER AND, AGAIN, A NUMBER IN THE SCID THERAPEUTIC AREA.

THESE ARE THE INVESTIGATORS THAT MANY OF US HAVE BECOME FAMILIAR WITH. THE INSTITUTIONS, NOW THIS IS IMPORTANT. SO SOME OF THESE ARE BEING RUN NOT ONLY BY PUBLIC INSTITUTIONS, BUT ALSO PRIVATE INSTITUTIONS ON THE BIOTECH SIDE. AND MORE IMPORTANTLY, THE FDA DESIGNATION. SO WE HAVE FDA ACCELERATED DESIGNATION UNDER RMAT FOR FOUR OF THESE, INCLUDING A BREAKTHROUGH DESIGNATION WHICH ACCELERATES NOT ONLY THE DISCUSSION WITH THE FDA, BUT ALSO COMMERCIALIZATION POTENTIAL IF, IN FACT, THE DATA READS OUT AS POSITIVE.

SO I THINK IT'S IMPORTANT TO PAUSE HERE JUST TO PROVIDE SOME FEEDBACK. ONE, MANY STUDIES IN RARE DISEASE, AND THERE'S PRECEDENCE FOR THIS, CAN BE APPROVED WITH PHASE 1, PHASE 2 DATA WITH CONFIRMATORY TRIALS THEREAFTER. SO WHEN WE START THINKING ABOUT OPTICS ON THESE TRIALS, IT'S IMPORTANT TO PREPARE THINKING ABOUT THE DATA READOUT AND, MORE IMPORTANTLY, SORT OF THE INFRASTRUCTURE THAT'S GOING TO BE REQUIRED TO SUPPORT THE COMMERCIALIZATION OF THESE THERAPIES.

GENERALLY, IN MY EXPERIENCE, IT TAKES

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ABOUT ONE TO TWO YEARS, MINIMUM OF ONE, MAYBE TWO YEARS TO PREPARE ALL OF THE INFRASTRUCTURE THAT'S REQUIRED TO GET DRUGS TO PATIENTS ONCE YOU GET FDA APPROVAL. ONE OF THE THINGS I'D LIKE TO PRESENT TO THE AAWG MAYBE MOVING FORWARD IS A COMMERCIAL LAUNCH STRATEGY. WHAT DOES THAT LOOK LIKE? AND SORT OF THE HIGH END COMPONENTS OF EVERYTHING THAT NEEDS TO BE PUT IN PLAY TO SUPPORT ALL THE WAY FROM MANUFACTURING, DISTRIBUTION, PROVIDING A SAFE AND EFFECTIVE USE OF THE DRUG, AND, MORE IMPORTANTLY, WHICH WE'LL TALK ABOUT IN A FEW MINUTES, IS THE POST-MARKETING SURVEILLANCE.

SO CIRM IS STRATEGICALLY ALIGNED WITH THE FDA AND NIH WITH RESPECT TO THE FOCUS ON RARE DISEASE. JUST GIVE YOU A COUPLE OF EXAMPLES. ONE, CERTAINTY ALIGNED WITH THE CENTER FOR BIOLOGICAL EVALUATION, RESEARCH THAT CBER WHICH EVALUATES ALL CELL AND GENE THERAPY SUBMISSIONS. THEY ARE EXPANDING OR POTENTIALLY EXPANDING THEIR RARE DISEASE WITH WHAT'S CALLED THE OPERATION WARP SPEED FOR CELL AND GENE THERAPIES. WE'RE OBVIOUSLY ALIGNED WITH CDER, WHO'S BEEN AN HEAVY HITTER, OF COURSE, ON THE SMALL MOLECULE SIDE WITH RESPECT TO RARE DISEASES. THERE'S A NEW INITIATIVE, AND THERE'S MORE INITIATIVES TO INCREASE THE SIZE OF

CDER TO APPROACH MORE NOVEL WAYS OF BRINGING RARE DISEASES OR AT LEAST THERAPIES TO RARE DISEASE POPULATIONS. AND MORE IMPORTANTLY, AS I'VE LEARNED RECENTLY, CIRM HAS JOINED THE ACCELERATING MEDICINES PARTNERSHIP. THIS IS A COLLABORATION, OF COURSE, THROUGH THE BESPOKE GENE THERAPY CONSORTIUM. THIS INCLUDES ABLA AND HER TEAM, OF COURSE, AND SHYAM. THIS IS A PUBLIC/PRIVATE PARTNERSHIP BETWEEN THE NIH, FDA, AND MULTIPLE PUBLIC AND PRIVATE ORGANIZATIONS.

POINT OF THIS SLIDE IS TO JUST SAY, HEY, LOOK. WE ARE ALIGNED WITH SOME PRETTY HEAVY HITTERS OUT THERE THAT ARE REALLY PUTTING A LOT OF EMPHASIS IN THE RARE DISEASE SPACE.

NOW, THE DELIVERY FOR THERAPIES FOR RARE DISEASE HAS BEEN UNIQUELY CHALLENGING. AND THERE ARE A NUMBER OF COMPONENTS TO A LAUNCH MECHANISM, IF YOU WILL. THERE'S CHALLENGES ON THE CLINICAL SIDE. MANY OF THE CLINICIANS HERE PROBABLY WOULD CONCUR THAT, ONCE THEY ARE EXPOSED TO A RARE DISEASE, ONE, THERE'S NOT A LOT OF INFORMATION THAT'S OUT THERE ABOUT THE DISEASE. THERE'S DIFFICULTY UNDERSTANDING SORT OF THE ENDPOINTS IF YOU WANT TO THINK ABOUT FROM A REGULATORY STANDPOINT. I DON'T WANT TO GO THROUGH ALL THESE. I WANT TO TOUCH ON A COUPLE OF

THINGS BECAUSE IT'S IMPORTANT WITH RESPECT TO CIRM'S FIVE-YEAR STRATEGIC PLAN. IF YOU THINK ABOUT CROSS FUNCTIONALLY OF ALL THE DEPARTMENTS, EACH DEPARTMENT IS PROPOSING OR EVEN IMPLEMENTING MECHANISMS RIGHT NOW THAT COULD FOR THE MOST PART HELP LIFT AND PROVIDE THE OPPORTUNITY FOR A SUCCESSFUL LAUNCH. AND EVEN IF YOU THINK ABOUT ACCESS AND AFFORDABILITY TO PATIENTS OUT THERE WHO A WAITING FOR THE COMMERCIALIZATION OF SOME OF THESE THERAPIES.

SO DATA GENERATION HAS ALWAYS BEEN DIFFICULT IN THE RARE DISEASE SPACE. LIMITED NUMBER OF PATIENTS. IF YOU THINK ABOUT PATIENT REGISTRIES, THAT'S A GREAT OPPORTUNITY FOR US TO CONSIDER ON THE ROADMAP ON THE RARE DISEASE SIDE. WE'LL TALK ABOUT THAT IN A FEW MINUTES. MANUFACTURING, CERTAINLY NOT MY AREA OF EXPERTISE. SHYAM COULD PROBABLY PROVIDE A NUMBER OF LECTURES, BUT THERE ARE OBVIOUSLY CHALLENGES FROM TAKING CLINICAL SCALE TO COMMERCIAL SCALE, CMC CONSIDERATIONS. OF COURSE, WE HAVE A NUMBER OF MANUFACTURING INITIATIVES ON THE STATE THAT COULD HELP LIFT SOME OF THOSE HURDLES.

REGULATORY, IT'S BECOMING MUCH BETTER. I THINK IN MY EXPERIENCE IN THE PAST, IT WAS DIFFICULT TO UNDERSTAND WHAT THE STANDARDS FOR APPROVAL WERE, WHAT THOSE PRIMARY AND SECONDARY ENDPOINTS WERE

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GOING TO BE. I THINK THAT'S GOTTEN MUCH BETTER OVER TIME. IN FACT, WE HAVE A COLLEAGUE WHO IS IN THE INDUSTRY WHO DOES HAVE DIRECT INTERACTION ON THE POLICY SIDE AND ON THE CLINICAL SIDE, WHO HAPPENS TO A CEO OF A BIOTECH COMPANY THAT IS NOW IN DISCUSSIONS, AND HOPEFULLY WE'LL BE ABLE TO WORK WITH THEM OR CONSIDERATION OF WORKING WITH THEM, BIOMARKERS, EARLY BIOMARKERS, SURROGATE BIOMARKERS THAT CAN BE USED FOR PRIMARY AND SECONDARY ENDPOINTS. AND I'M SURE ABLA AND HER TEAM COULD SPEAK AT LENGTH ON THAT TOPIC.

THE AREA THAT I WANT US TO CONSIDER TODAY, WHICH, AGAIN, CAN BE PART OF THE ROADMAP, PARTICULARLY FOR RARE DISEASES, IS THE POST-MARKETING REQUIREMENTS. AND THE COMBINATION OF NOT ONLY THE POST-MARKETING REQUIREMENTS, BUT ALSO NOW THE PAYER REIMBURSEMENT. SO HERE'S WHAT'S HAPPENING REAL-TIME. IT'S QUITE INTERESTING.

POST-MARKETING IS NOT NEW. THOSE MANDATES HAVE BEEN REQUIRED FOR MANY ACCELERATED THERAPIES, WHETHER IT'S SMALL MOLECULE OR EVEN CELL AND GENE THERAPY. WHAT'S INTERESTING NOW IS THE POST-MARKETING SURVEILLANCE HAS BECOME SO CRITICAL, AND I'LL TALK ABOUT THIS IN A FEW MINUTES. THE PAYER INFORMATION THAT IS COMING OUT OF THAT

CLINICAL DATASET ON THE POST-MARKETING SIDE IS ABSOLUTELY CRITICAL FOR REIMBURSEMENT TO THE PUBLIC AND PRIVATE PAYERS. SO MANUFACTURERS ARE ABSOLUTELY DEPENDENT AND GEARING UP RIGHT NOW TO SET UP THESE SYSTEMS THAT ARE ROBUST TO SUPPORT THE POST-MARKETING REQUIREMENTS FOR THE FDA AND, MORE IMPORTANTLY, THE REQUIREMENTS FROM A PAYER LANDSCAPE, AND THAT'S SOMETHING THAT'S NEW.

SO WHY POST-MARKETING COMMITMENTS ARE IMPORTANT. AGAIN, PER FDA, ALL COMMERCIALLY APPROVED CELL AND GENE THERAPIES REQUIRE THE MANUFACTURER TO OVERSEE POST-MARKETING COMMITMENTS FOR UP TO 15 YEARS. AND THAT'S A BIG ASK. THAT'S A BIG ASK FOR SMALL BIOTECH, AND IT'S CERTAINLY A BIG ASK FOR POTENTIAL ACADEMIC INSTITUTIONS WHO DO WANT TO FILE A BLA.

IN ADDITION, AS I MENTIONED EARLIER, MANDATED SAFETY REPORTING FOR MANY OF THE POST-MARKETING TRIALS. POST-MARKETING DATA HAS BECOME INSTRUMENTAL FOR REIMBURSEMENT THROUGH THE VALUE-BASED AGREEMENTS WITH PAYERS. AND MANY OF YOU ON THE AAWG HAVE EXPERIENCE WITH THIS. I THINK I MENTIONED IN OUR PREVIOUS DISCUSSIONS ABOUT WHAT THOSE VALUE-BASED AGREEMENTS LOOK LIKE. AGAIN, THEY ARE SORT OF PROPRIETARY. THEY ARE. WE'RE GETTING

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INTEL ON A DAILY BASIS IN TERMS OF WHAT THAT'S GOING TO LOOK LIKE. AND, AGAIN, HOW THIS FEEDS INTO THE IMPORTANCE OF THE POST-MARKETING SURVEILLANCE.

ANOTHER THING WE NEED TO THINK ABOUT, AND I'LL THROW IT OUT THERE, IS THE PATIENT JOURNEY. WE TALKED ABOUT THIS DURING THE PATIENT SUPPORT PROGRAM AND THE INFRASTRUCTURE THAT'S NEEDED JUST TO GET THEM THROUGH THE CLINICAL TRIALS. NOW THERE'S ANOTHER COMPONENT THAT CIRM SHOULD CONSIDER, AAWG, IS WHAT'S THAT LIFT FROM A PATIENT SUPPORT STANDPOINT? MORE IMPORTANTLY, THE PATIENT JOURNEY IN THE POST-MARKETING LANDSCAPE. SO, AGAIN, ALL OF THIS IS SORT OF HAPPENING REAL-TIME. THERE'S SOME REALLY GOOD WHITE PAPERS I CAN DISSEMINATE LATER OF THEORETICAL OPPORTUNITIES FOR WHERE THERE CAN SUSTAINABILITY IN THE SPACE.

THIS IS SORT OF A HEAVY SLIDE. THIS SLIDE SORT OF ADDRESSES COMPONENTS OF OUR FIVE-YEAR STRATEGIC PLAN. SO UNDER MEDICAL AFFAIRS, WE ARE LOOKING TO IMPLEMENT, NOT ONLY REAL-WORLD DATA, BUT ALSO HEALTH ECONOMICS OUTCOMES AND RESEARCH. SO THERE'S AN OPPORTUNITY FOR US HERE TO CONSIDER A POTENTIAL FUNDING MECHANISM FOR OUR AWARDEES ON THE POST-MARKETING SURVEILLANCE. AND WE CAN TALK ABOUT THIS IN A FEW MINUTES IN MORE DETAIL. BUT THERE ARE

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A NUMBER OF ADDITIONAL OPPORTUNITIES THAT WE CAN THINK THROUGH. AND THAT IS BRIDGING THE GAP TO REAL-WORLD DATA.

SO I DON'T THINK IT'S ANY SURPRISE TO THE AAWG, BUT WE DON'T HAVE ACCESS TO A LOT OF DATA. SO THIS COULD BE A BRIDGE POTENTIALLY IF IT'S ENDORSED IN THE ROADMAP WHERE WE CAN START BUILDING THE INFRASTRUCTURE FOR, NOT ONLY THE REAL-WORLD DATA, BUT ACTUALLY THE HEOR, THE HEALTH ECONOMICS OUTCOMES AND RESEARCH COMPONENT.

SO I JUST WANTED TO SHARE THIS SLIDE THAT WE ARE THINKING THROUGH NOT ONLY THE OPPORTUNITIES FROM A PATIENT SUPPORT STANDPOINT -- EXCUSE ME -- FROM A SURVEILLANCE STANDPOINT, REAL-WORLD DATA RESEARCH TO DETERMINE THE GREATEST IMPACT OF SPECIFIC POPULATIONS WITHIN THAT DATASET, AND, MORE IMPORTANTLY, THE REIMBURSEMENT STRUCTURE INFRASTRUCTURE.

NOW, ALSO WHAT'S INTERESTING, YESTERDAY WE HAD OUR STEERING COMMITTEE FOR THE ALPHA CLINICS. AND I SURE HOPE NOBODY TEED ANYBODY UP, BUT VERY FIRST CONVERSATION WAS REALLY ABOUT POST-MARKETING SURVEILLANCE. AND ALREADY MANY OF THE ACADEMIC INSTITUTIONS ARE STARTING TO THINK THROUGH THAT PROCESS FROM A PHARMOCOVIGILANCE STANDPOINT, WHAT

THE OPERATIONS THAT ARE GOING TO BE NEEDED TO SUPPORT THIS ENTIRE MECHANISM.

SO, AGAIN, THIS SLIDE JUST SHOWS YOU SORT OF WHAT WE ARE THINKING THROUGH ON THAT PHASE IV LONGITUDINAL, THE OPPORTUNITIES, AND SOME OF THE INDUSTRY STANDARD, PAYER STANDARDS, SORT OF METRICS THAT ARE REQUIRED AND ANALYSES FROM A POST-MARKETING STANDPOINT.

LET ME PAUSE RIGHT NOW BECAUSE I KNOW THERE'S A COUPLE HANDS THAT HAVE COME UP. I KNOW I'M MOVING VERY QUICKLY. BUT, FIRST, IF IT'S OKAY, LET ME TAKE A COUPLE QUESTIONS AT THIS TIME.

VICE CHAIR BONNEVILLE: AMMAR HAS HIS HAND RAISED.

DR. QADAN: THANK YOU, SEAN. THIS IS REALLY GREAT TO SEE. I THINK IT WAS TWO MEETINGS AGO WHEN ABLA PRESENTED THE CLINICAL TRIAL PROGRAM. I ASKED THE QUESTION AROUND HOW WE ARE GATHERING SOME OF THAT DATA, ESPECIALLY FOR HEALTH ECONOMICS AND OUTCOMES RESEARCH. AND I REMEMBER SHE SAID IN ALL OF THOSE STUDIES WE'RE USING A THIRD PARTY TO COLLECT THAT DATA.

SO TO THAT, MAYBE NOW, IF WE THINK WE WANT FOCUS ON POST-MARKETING CONSIDERATIONS FOR THE SAME REASON, IS THERE A POSSIBILITY OF HAVING LIKE

EXPERTISE, LIKE HEALTH ECONOMICS AND OUTCOMES RESEARCH HEAD COUNT, WHO COULD BE PART OF THE TEAM AND OVERSEE ALL OF THOSE THINGS SO THAT WE CAN HAVE AT LEAST A CENTRAL RESOURCE FOR ALL OF THAT? THANK YOU.

DR. TURBEVILLE: LET ME RESPOND. GOOD QUESTION. SO WE HAVE BEEN THINKING THROUGH -- WE'RE ONE YEAR DEEP INTO MEDICAL AFFAIRS. BUT THE ANSWER IS WE CAN DO THIS, THROUGH AAWG'S RECOMMENDATION, WE COULD DO THIS TWO WAYS. ONE IS POTENTIALLY BRINGING IT IN-HOUSE WITH AN FTE THAT'S GOT THE EXPERIENCE ON NOT ONLY REAL-WORLD DATA, BUT HEOR AND POTENTIALLY A COMBINATION OF EVEN A CONSULTANT AS WELL.

SO THIS IS ONE OF THE HEAD COUNTS THAT MARIA MILLAN AND I DISCUSSED THAT IS NEEDED, PARTICULARLY ALL THE ENERGY THAT'S GOING INTO THIS RIGHT NOW. AND IT IS A GREAT OPPORTUNITY FOR US TO BRIDGE THE GAP TO ALL THOSE DATASETS POTENTIALLY.

I KNOW MANY OF YOU WORK IN THIS SPACE ON THE BIG DATA. THERE ARE LOTS OF DATASETS OUT THERE THAT WE HAVEN'T EVEN TAPPED INTO THAT ARE AN OPPORTUNITY FOR THE AAWG TO CONSIDER FROM A ROADMAP PERSPECTIVE. THANK YOU FOR THAT QUESTION.

VICE CHAIR BONNEVILLE: SCOTT, DID YOU WANT TO SAY SOMETHING?

MR. TOCHER: SURE. THANKS. SORRY TO INTERRUPT. JUST WITH THE UPDATED DIAL-IN INFORMATION, JUST WANTED TO DRAW THE PUBLIC'S ATTENTION IF THERE'S ANY PUBLIC THAT WANTS TO PARTICIPATE DURING THE PUBLIC COMMENT SESSION, WE HAVE UPDATED THAT CONTACT INFORMATION ON THE AGENDA ON THE WEBSITE. SO BE SURE AND UPDATE THAT INFORMATION, AND WE'LL WELCOME YOUR PUBLIC COMMENT. THANKS, MARIA.

DR. TURBEVILLE: THANK YOU, SCOTT.

VICE CHAIR BONNEVILLE: ARE THERE ANY OTHER QUESTIONS FROM THE GROUP? OKAY, SEAN. YOU WANT TO CONTINUE.

DR. TURBEVILLE: ALL RIGHT. THANK YOU.

SO I KNOW THIS IS SORT OF A TRICKY PRESENTATION SINCE WE ARE TRYING TO PRESENT AN OPPORTUNITY HERE, AND THERE'S LOT OF GRANULARITIES WHEN IT COMES TO POST-MARKETING SURVEILLANCE. I DO WANT TO FINALIZE WITH A COUPLE SLIDES AND THEN REALLY OPEN IT UP FOR DISCUSSION. LIKE TO GET THE INPUT FROM THE AAWG ALL THE WAY FROM THE CLINICAL SIDE AND, OF COURSE, THE PATIENT ADVOCACY SIDE REALLY FROM A PATIENT JOURNEY STANDPOINT, SEE WHERE ALL THESE PATIENTS ARE GOING THROUGH THE ENTIRE JOURNEY.

SO WHY IT'S IMPORTANT TO POTENTIALLY FUND POST-MARKETING COMMITMENTS FOR CIRM PROGRAMS. SO WHAT I MENTIONED EARLIER IS WE HAVE THE OPPORTUNITY FOR REAL-WORLD EVIDENCE IS PLAYING AN INCREASING ROLE IN HEALTHCARE DECISION. IT PROVIDES CLINICAL EVIDENCE REGARDING THE USAGE, THE BENEFITS, AND RISKS OF A MEDICAL PRODUCT.

THE SECOND, OF COURSE, IS THE HEALTH ECONOMICS AND OUTCOMES RESEARCH, AGAIN, GENERATES THE EVIDENCE FOR THE VALUE OF NEW THERAPY FOR REIMBURSEMENT AND HEALTHCARE PAYERS.

SO THIS IS ONE COMPONENT THAT WE WERE THINKING THROUGH FROM THE MEDICAL AFFAIRS STANDPOINT THAT WE WOULD LIKE TO GET INPUT OF POTENTIALLY INCLUDING THIS IN THE ROADMAP. NOT ONLY GIVEN THE IMPORTANCE OF THE POST-MARKETING SURVEILLANCE, THERE'S A NUMBER OF OPPORTUNITIES HERE THAT SORT OF OVERLAP.

ONE, WE'VE GOT AN EXPANSION OF THE ALPHA CLINICS, AND IT WAS CONFIRMED YESTERDAY THAT THEY'RE THINKING THROUGH THIS AS WELL.

TWO, IF YOU THINK ABOUT TRUE ACCESS AND AFFORDABILITY, THINK ABOUT THE COMMUNITY CARE CENTERS OF EXCELLENCE THAT WE'RE TRYING TO PUT OUT THERE, RIGHT. WE'RE AHEAD OF SCHEDULE WITH THAT.

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WE JUST HAD OUR THIRD LISTENING SESSION, WHICH WE'LL GET A DEBRIEF MAYBE IN THE NEXT MEETING, BUT HOW CAN THEY SORT OF POSITION THEMSELVES TO BE ABLE TO PARTICIPATE IN THE POST-MARKETING REQUIREMENTS FOR THE PATIENTS. THESE ARE BIG ASKS. IF YOU THINK ABOUT THE NUMBER OF SICKLE CELL PATIENTS THAT ARE GOING TO BE REQUIRED TO GO THROUGH POST-MARKETING, REALLY DIFFICULT TO GET ALL THE PATIENTS TO THE CENTERS OF EXCELLENCE. AND SO THIS TIES IN NICELY WITH OUR VISION OF WHAT THE COMMUNITY CARE CENTERS OF EXCELLENCE, WHAT ARE THOSE DELIVERABLES THAT THEY CAN PROVIDE FOR PATIENTS AND ALSO ON THE CLINICAL SIDE.

AND, AGAIN, JUST TO REITERATE, IT GIVES US THAT FOUNDATION POTENTIALLY TO BUILD THAT REAL-WORLD EVIDENCE AND HEOR. AND SO BEFORE I OPEN IT FOR QUESTIONS AND COMMENTS, I DO WANT TO JUST FOR THIS SLIDE GIVE A SUMMARY OF THE ACCESS AND AFFORDABILITY TOPICS THAT WE TALKED ABOUT SO FAR. SO BACK IN THE DAY, WE TALKED ABOUT, AND WE CONTINUE, ON THE PATIENT SUPPORT SERVICE MODEL. AND I WOULD EVEN ARGUE NOW THAT PATIENT SUPPORT SERVICES IS EVEN MORE IMPORTANT BECAUSE NOW THEY'RE TAKING PATIENTS TO THAT POST-MARKETING STUDY AS WELL.

WE TALKED ABOUT NEW PAYER MODELS IN THE

PAST, THE VALUE-BASED AGREEMENTS. THERE'S A NUMBER OF THEM, VERY TECHNICAL. AND, OF COURSE, THOSE ARE PROPRIETARY WITH PAYERS, BUT THOSE ARE ABSOLUTELY INSTRUMENTAL. IT'S SOMETHING THAT, IF, IN FACT, ANY OF OUR ASSETS GO TO MARKET AUTHORIZATION, IT'S SOMETHING THAT EITHER THE MARKETING AUTHORIZATION HOLDER WILL HAVE TO DEAL WITH OR POTENTIALLY WE CAN PROVIDE SOME GUIDANCE.

WE TALKED LAST WEEK OR MAYBE THE LAST PRESENTATION ABOUT INPATIENT TO OUTPATIENT SETTING, PARTICULARLY ON THE CAR-T SIDE. HARLAN PROVIDED GREAT GUIDANCE ON THIS. THERE IS A RACE TO GET OUT THERE TO THE COMMUNITY.

WE TALKED, BRIEFLY DISCUSSED A LITTLE BIT ABOUT STATE AND FEDERAL POLICY. WE CAN GO INTO MUCH MORE DETAIL. IN FACT, WE'RE DOING SOME MORE DUE DILIGENCE ON THAT LANDSCAPE, BUT CERTAINLY THE CALIFORNIA CARE EQUITY ACT IS SOMETHING THAT WE CAN CERTAINLY REVISIT THAT WOULD HAVE A DIRECT IMPACT FOR PATIENTS.

WE HAVE THE EXPANSION OF THE ALPHA CLINICS THAT'S JUST TAKEN PLACE. AND NOW WE ARE STARTING TO THINK THROUGH THAT CONCEPT PLAN FOR THE COMMUNITY CARE CENTERS OF EXCELLENCE.

THIS DISCUSSION SORT OF TEES UP THAT

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REAL-WORLD DATA AND THE OPPORTUNITY TO THINK ABOUT A FUNDING MECHANISM, NOT ONLY IN RARE DISEASE, BUT OUTSIDE OF RARE DISEASE, FOR REAL-WORLD DATA IN THAT POST-MARKETING SURVEILLANCE. AGAIN, THIS TIES INTO THE HEALTH ECONOMICS AND RESEARCH THAT WE ARE LOOKING FORWARD TO PUTTING IN PLAY HERE FROM A MEDICAL AFFAIRS STANDPOINT.

AND THEN, FINALLY, WE HAVE ONE MORE PRESENTATION BEFORE I OPEN IT UP FOR DISCUSSION AND COMMENTS. AND THAT IS A FASCINATING SPACE THAT ALSO, AS J.T. MENTIONED, IS EVOLVING REAL-TIME. AND THAT'S THE COVERAGE ANALYSIS, ACCESS AND APPEALS, THE COPAYS, AND THE RISK POOLS. I KNOW THAT'S A LOT, BUT WE ARE TRYING TO JUST TEE UP THE NEXT PRESENTATION THAT I THINK IS GOING TO BE IMPORTANT WHEN WE THINK ABOUT LOCKING DOWN OUR ACCESS AND AFFORDABILITY ROADMAP TO BE PRESENTED, AGAIN, TO THE AAWG AND THEN TO HOPEFULLY THE ICOC FOR A BLESSING FOR POTENTIAL FUNDING MECHANISMS.

SO WITH THAT, I KNOW THAT'S A LOT OF INFORMATION, BUT I WANT TO PAUSE HERE. WANT TO THANK YOU. AND OPEN THIS UP FOR DISCUSSION FOR ALL OUR CROSS-FUNCTIONAL COLLEAGUES.

VICE CHAIR BONNEVILLE: TED HAS HIS HAND RAISED.

DR. GOLDSTEIN: THANKS. SO I THINK THIS WAS A GOOD INTRODUCTION AND MAYBE FOR THE PRESENTATION AFTER THAT BECAUSE I THINK THE PAYER PRESENTATION IS A GREAT IDEA FOR THE NEXT DISCUSSION. BUT ONE OF THE THINGS IS THERE'S SOME PRAGMATICS ABOUT STEM CELL THERAPIES THAT MIGHT BE USEFUL FOR US TO GET BRIEFED ON.

SO AS I UNDERSTAND IT, AND PLEASE, THERE ARE PEOPLE ON THIS CALL WHO KNOW SO MUCH MORE ABOUT IT THAN I TO CORRECT, THAT ESSENTIALLY WHAT WE'RE DOING HERE IS PRECISION THERAPEUTICS WHERE WE ARE LOOKING AT THE ACTUAL GENETIC MALFUNCTION THAT'S CAUSING THESE RARE DISEASES IN MANY CASES. AND WE HAVE THREE BROAD CATEGORIES OF THERAPIES, THERAPEUTIC WAYS OF FIXING THE BROKEN CELLS, TO PUT IT IN KIND OF LAYMAN'S LANGUAGE, A LENTIVIRAL VECTOR MODIFICATION, A CRISPR MODIFICATION, OR A MODIFICATION OR CREATING A GENERIC CELL THAT IS PORTABLE TO MANY PEOPLE.

THE FIRST TWO THERAPIES, WE ARE TAKING THE PATIENT'S OWN CELLS, MODIFYING THEM, AND PUTTING THEM BACK. IN THE THIRD ONE, WE'RE TAKING THINGS FROM A DONOR OR MANUFACTURED THROUGH SOME PROCESSES. I BELIEVE THAT ONE OF THE REASONS WHY WE ARE BEING ASKED TO DO THE KIND OF POST-MARKETING SURVEILLANCE

IS WE DO NOT UNDERSTAND THE FULL IMPLICATIONS OF THOSE THERAPIES, THAT THERE ARE THE SIDE EFFECTS POSSIBLE, OFF-TARGET EFFECTS, AND SO ON. AND, OF COURSE, THE NOVELTY OF THIS NEW CLASS OF THERAPY, WE NEED TO LOOK DEEPER.

WOULD IT BE POSSIBLE FOR US TO GET PERHAPS A MORE RIGOROUS DISCUSSION ABOUT THIS SO THAT WHEN WE PROVIDE OUR FEEDBACK AND RECOMMENDATIONS, WE UNDERSTAND WHICH OF THESE DIFFERENT CLINICAL TRIALS THAT WE ARE SUPPORTING SHOULD BE FOCUSED ON SO THAT WE CAN HELP DETERMINE WHAT ARE THE RIGHT IMPLICATIONS SO THAT WE HAVE A BALANCED PORTFOLIO OR WE ARE NOT. WE DECIDE TO FOCUS ON ONE AVENUE OF THOSE THREE.

DR. TURBEVILLE: YEAH. VERY GOOD. WE HAVE DONE DUE DILIGENCE ON ALL OF THE POST-MARKETING REQUIREMENTS THAT HAVE TAKEN PLACE TO DATE FOR APPROVED CELL AND GENE THERAPIES. WHAT I CAN DO AFTER THIS PRESENTATION, IF IT'S OKAY WITH THE VICE CHAIRMAN, IS SEND THAT OUT TO THE ENTIRE TEAM. IT WAS ORIGINALLY IN THIS DISCUSSION, BUT I THOUGHT IT WAS A LITTLE BIT TOO HEAVY. BUT, YEAH, I THINK THAT WOULD BE A GOOD STARTING POINT JUST TO SEE WHAT THE BENCHMARKS ARE NOW FOR CELL AND GENE THERAPIES THAT HAVE BEEN APPROVED.

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DR. GOLDSTEIN: BUT ALSO TO UNDERSTAND WHAT ARE THE CAUSES AND WHY, WHY CELL AND GENE THERAPIES ARE DIFFERENT.

DR. TURBEVILLE: CERTAINLY. THERE'S A LOT OF -- THAT'S REALLY -- I THINK YOU BRING UP A GREAT POINT. THAT'S ONE OF MAIN MECHANISMS OF THE POST-MARKETING SURVEILLANCE IS THAT LONG-TERM SAFETY, EFFICACY, AND DURABILITY. YEAH, I HEAR YOU. I THINK THAT MIGHT BE HELPFUL.

DR. GOLDSTEIN: THANK YOU.

VICE CHAIR BONNEVILLE: TED, WERE YOU ASKING HOW IT RELATES, THEN, TO WHAT WE ARE CURRENTLY FUNDING, WHAT THAT INTERSECTION IS?

DR. GOLDSTEIN: YES. THERE'S KIND OF A SWEET SPOT IN TRADITIONAL DRUG THERAPIES, LARGE ENOUGH MARKET AND ACCESSIBILITY AND SAFETY AND SO ON. WE ARE NOT IN THAT SWEET SPOT. WE ARE EXPANDING BEYOND THAT WITH THIS NEW CLASS OF THERAPIES.

VICE CHAIR BONNEVILLE: THANKS, TED. HARLAN.

DR. LEVINE: GOOD AFTERNOON. I HAVE MOSTLY QUESTIONS, AND MAYBE THERE'S A COMMENT INHERENT IN THE QUESTION. SO I THINK THESE ARE BEING HELD SEPARATELY BECAUSE THE VOLUMES ARE SO LOW

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BEFORE APPROVAL AND THEY'RE SO TAILORED TO INDIVIDUALS, THAT I THINK THERE'S JUST ADDED CONCERN THAT, AS THE NUMBERS EXPAND AND WE GET MORE -- THERE'S A BALANCE BETWEEN TRYING TO GET SOMETHING TO MARKET FOR BENEFIT VERSUS TRYING TO RECOGNIZE IN THESE RARE CONDITIONS OR PERSONALIZED CONDITIONS THAT YOU CAN'T GET THE NUMBERS THAT YOU GET FOR TRADITIONAL TRIALS. THEREFORE, THERE'S MORE EMPHASIS ON POST-MARKETING OR POST-APPROVAL RECORD COLLECTION.

SO, ANYWAY, THAT'S MY COMMENT THAT I HOPE IS CORRECT AND COULD BE WRONG.

MY QUESTIONS FOR CONTEXT ARE, ONE, IF WE DON'T GET INVOLVED IN THIS, HOW WOULD FUNDING OCCUR? BECAUSE IT SEEMS LIKE POST-MARKETING, THERE'S A MARKET OUT THERE THAT WOULD BE WILLING TO INVEST AND DO THIS. SO THAT'S QUESTION NO. 1.

QUESTION NO. 2 IS IN JOE BIDEN'S BUDGET THAT CAME OUT THIS WEEK, HE TALKED ABOUT GETTING STRICTER ACROSS THE BOARD ON POST-MARKETING DATA COLLECTION AND THAT THERE WOULD BE THINGS THAT WOULD BE CONDITIONALLY APPROVED; BUT IF THE MARKETING WASN'T DONE, THERE WOULD BE PENALTIES OR WITHDRAWAL OF APPROVAL FOR PAYMENT. HOW DOES THAT RELATE TO THIS TOPIC IF AT ALL? THAT'S WHERE --

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AND THEN MY THIRD COMMENT IS I'M NOT EXPERT AT THIS EITHER. SO I'M SURE THERE'S A THOUSAND OTHER QUESTIONS THIS GROUP SHOULD BE CONSIDERING. WHAT ARE SOME OF THE OTHER GEOPOLITICAL ISSUES THAT ONE MIGHT SAY THAT WE SHOULD BE TAKING INTO ACCOUNT AS WE ARE THINKING ABOUT WHAT CIRM'S RESPONSIBILITIES ARE?

DR. TURBEVILLE: LET ME SEE IF I CAN PIECEMEAL EACH ONE OF THOSE. THANK YOU, HARLAN, FOR THE QUESTIONS. ONE, YEAH. FOLKS ARE REALLY GEARING UP. I THINK WE ARE INTERACTING OR JUST TRYING TO GET AS MUCH INTEL AS POSSIBLE ABOUT THE NEED NOW TO REALLY RAMP UP FROM THE POST-MARKETING SURVEILLANCE SERVICES. AGAIN, IT'S BECOMING EVEN MORE CRITICAL NOW BECAUSE THEY WERE NOT REALLY PREPARED FOR THE REIMBURSEMENT DATA THAT'S SO CRITICAL THAT'S COMING OUT OF THAT POST-MARKETING, AND THIS TIES INTO THE SORT OF BIDEN DISCUSSION WHERE WE ARE NOW SEEING LITERALLY IN THE LAST WEEK OR TWO HOW IMPORTANT THE METHODOLOGY AND THE INFRASTRUCTURE THAT'S REQUIRED FOR THESE POST-MARKETING TRIALS. IT IS WHAT IT IS. AND THEY WANT ROBUST, METHODOLOGICALLY SOUND DATA, AND HERE'S SORT OF WHERE WE MIGHT BE ABLE TO BRIDGE THE GAP.

MANY SMALL BIOTECH COMPANIES, AND I'VE

LAUNCHED A PHASE IV TRIAL, THEY ARE HEAVY. IT'S A HEAVY ASK FROM A FINANCIAL AND FROM AN OPERATIONAL STANDPOINT. AND IF WE THINK ABOUT EVEN THE ACADEMIC INSTITUTIONS THAT ARE THINKING ABOUT TAKING THIS TO A BLA, YOU REALLY HAVE TO START THINKING TO WHERE WE MIGHT BE ABLE TO FILL THE GAPS FROM AN OPERATIONAL STANDPOINT.

IT IS AN OPPORTUNITY FOR US FOR THE COMMUNITY CARE CENTERS OF EXCELLENCE, INCLUDING THE ALPHA CLINICS. AND IT'S OPEN FOR DISCUSSION. THINGS ARE HAPPENING REAL-TIME. SO THE DISCUSSIONS WE'RE HAVING WITH PAYERS, AS FAR AS I CAN TELL, IS ALL ABOUT HOW ROBUST THAT DATA IS FROM A POST-MARKETING STANDPOINT. THIS GETS KIND OF INTO METHODOLOGY. AND I DON'T KNOW IF I'M ANSWERING THE THIRD QUESTION, BUT I USED THE TERM "TSUNAMI" IN THE PAST IN TERMS OF NOT ONLY THE APPROVALS THAT ARE TAKING PLACE IN THE FDA, BUT NOW THERE'S SORT OF A TSUNAMI OF SETTING UP THIS INFRASTRUCTURE TO FILL ALL THE REQUIREMENTS, NOT ONLY FROM, AGAIN, FDA'S COMPETENT AUTHORITIES REQUIREMENTS, BUT ALSO FROM THE PAYERS REQUIREMENTS FOR EFFICACY, SAFETY, AND DURABILITY OF THE THERAPIES.

LET ME SEE IF I ANSWERED THE THIRD QUESTION OKAY, HARLAN, IF I ADDRESSED THAT.

DR. LEVINE: IT'S ALL RIGHT. MOOT ISSUE. I DIDN'T KNOW WHAT I WAS ASKING. I JUST WANTED TO KIND OF GET A BROADER VIEW OF THE THINGS THAT WE'RE CONSIDERING. I THINK THAT WAS HELPFUL.

I DO THINK -- I JUST CAN'T SEE HOW THIS IS GOING TO PLAY OUT. THE DRUGS COMING OUT HAVE SOME ASTRONOMICAL PRICE TAGS. I THINK THE PAYERS PLAY AN IMPORTANT ROLE IN TRYING TO CONTROL COST. I DON'T THINK AT THE END OF THE DAY THEY SHOULD BE THE FINAL ARBITER OF WHETHER SOMETHING HAS VALUE OR NOT. SO I DON'T WANT TO GIVE IN TO THE CURRENT DEFAULT, WHICH THE PAYERS ARE GOING TO DECIDE WHETHER OR NOT AND HOW MUCH TO PAY FOR THIS. THIS IS LIKE A NEW WORLD FOR US.

2017, ONE NEW DRUG HAD A PRICE OVER $200,000. THIS YEAR SIX OUT OF EIGHT OF THE NEW DRUGS HAVE PRICES OVER 200,000, AND WE'RE NOT EVEN TALKING ABOUT THE TYPE OF DRUGS WE'RE TALKING ABOUT HERE, WHICH ARE GENE THERAPIES AND CELL-BASED THERAPIES.

SO I THINK WE SHOULD BE VERY THOUGHTFUL ABOUT WHAT'S OUR POINT OF VIEW POLITICALLY ON WHO SHOULD BE DETERMINING THE PRICES OF THESE THINGS AND WHETHER OR NOT THEY'RE PAID FOR OR NOT.

VICE CHAIR BONNEVILLE: AGREE COMPLETELY.

SOMETHING YOU WERE SAYING EARLIER, HARLAN, ABOUT WHOSE RESPONSIBILITY IS IT, IS THIS A CIRM PROBLEM FOR THE POST-MARKETING DATA AND SETTING UP THOSE SYSTEMS AND PAYING FOR IT. SOMETHING TO CONSIDER IS BY THE TIME WE MOVE FORWARD IN THIS DIRECTION AND THEN IF IT'S A 15-YEAR FOLLOW-UP, CIRM MAY NOT BE AROUND. AND WHO KNOWS WHAT THAT SORT OF LANDSCAPE IS AND WHERE WE ARE IN OUR FUTURE. SO WHATEVER GETS SET UP OR WHATEVER WE FUND HAS TO HAVE THE ABILITY TO SUSTAIN ITSELF, WHETHER IT'S PLATFORMS OR TECHNOLOGIES THAT WE ENABLE JUST FROM A PRACTICAL PERSPECTIVE AND WHAT DOES THAT MEAN.

SO THAT REALLY SHOULD PLAY INTO THE CONVERSATION WHEN WE START TALKING MORE ABOUT HOW WE FUND AND WHAT WE ARE FUNDING.

DR. TURBEVILLE: IF I MIGHT OPINE. HARLAN, TO BE HONEST WITH YOU AND AMMAR, WHO IS OUR MARKET ACCESS EXPERT, PROBABLY COULD OPINE HERE. THOSE DISCUSSIONS ARE TAKING PLACE WITH THE PAYERS IN TERMS OF WHAT THOSE CLINICAL ENDPOINTS LOOK LIKE. AND THOSE ARE, OF COURSE, PROPRIETARY. AND WE'VE EVEN ASKED CMS TO HAVE MORE AUTHORITY IN TERMS OF WHAT'S REQUIRED FOR REIMBURSEMENT.

SO, YEAH, PAYERS ARE MAKING THOSE NEGOTIATIONS WITH THE MARKETING AUTHORIZATION, THE

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MANUFACTURER WHAT'S VALUE. AND SO THOSE DISCUSSIONS ARE TAKING PLACE. I DON'T KNOW IF AMMAR HAS ANY COMMENTS ON THIS.

DR. QADAN: I UNDERSTAND EXACTLY HARLAN'S CONCERNS BECAUSE YOU COULD GO EASILY INTO A RABBIT HOLE WITH ALL THE THINGS THAT PAYERS PUT IN PLACE. I WOULD FAIRLY BALANCE THAT BY SAYING THAT, EVEN IN HEALTHCARE SYSTEMS OUTSIDE THE U.S. WHERE PAYERS ARE INTEGRATED WITHIN THE HEALTHCARE SYSTEM LIKE IN THE UK AND FRANCE AND MANY OTHER COUNTRIES, WE HAVE SEEN THAT THEY'RE PLAYING AN IMPORTANT ROLE. THEY NEED SUCH TYPE OF DETAIL IN ORDER TO MAKE DECISIONS.

SO IT'S NOT UNIQUE TO WHAT WE SEE DEFINITELY IN THE U.S. IT IS A GLOBAL TREND. AND WHAT WE HAVE SEEN GENERALLY, EVEN OUTSIDE THE U.S., IS THAT THERE IS A FASTER ADOPTION OF SOME OF THOSE INNOVATIONS, MORE THAN THE U.S. STILL THEY REQUIRE MANY OF THAT DATA TO MAKE THOSE FUNDING DECISIONS TO START WITH.

DR. LEVINE: TO GIVE A LITTLE COLOR TO MY COMMENT, I'M NOT SAYING THERE'S A YES OR A NO OR IT'S A BLACK-AND-WHITE ISSUE. I JUST KNOW THAT I'VE BEEN IN THE BUSINESS FOR 30 YEARS, AND I KNOW THAT PROZAC WASN'T COVERED AS A FIRST-LINE THERAPY UNTIL IT WAS PROVEN THAT YOU HAD FEWER ER VISITS AND

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BETTER COMPLIANCE THAN YOU DID WITH THE TRADITIONAL ONES THAT WERE SO MUCH CHEAPER THAN OVERALL TOTAL COST TO CARE. SO THAT GOT DELAYED SEVERAL YEARS. OBESITY SURGERY WASN'T COVERED FOR YEARS AFTER IT WAS PROVEN TO BE MEDICALLY HELPFUL. THERE'S STILL STEP THERAPY GOING ON IN CANCER CARE WHICH WE KNOW IS COUNTERPRODUCTIVE.

SO MY POINT ISN'T THAT ONE CONSTITUENT OR ONE STAKEHOLDER SHOULD BE LEFT OUT VERSUS ANOTHER, ONLY THAT WE TEND TO DEFER THESE DECISIONS TO PAYERS BECAUSE IT'S SO IMPORTANT THAT THEY GET PAID FOR. BUT I THINK WE'RE JUST IN A NEW WORLD, THAT WE WANT TO NOT LET -- THE PAYER'S APPROACH HAS ALWAYS BEEN TO GO SLOW BECAUSE IT'S TO THEIR BENEFIT.

BY THE WAY, I'VE WORKED FOR PAYERS FOR OVER TEN YEARS. I DON'T WANT TO BE HYPOCRITICAL. I'VE BEEN PART OF THAT ECOSYSTEM. BUT I'M JUST SAYING WE SHOULD BE THOUGHTFUL ABOUT DO WE WANT TO DEFAULT TO THE CURRENT PATTERNS, OR DO WE WANT TO SET UP WHAT OCCURS IN THESE OTHER GLOBAL ENVIRONMENTS WHERE I THINK THEY'RE BETTER INTEGRATED BETWEEN ETHICISTS AND PATIENTS AND GOVERNMENT, PHYSICIANS, AND PAYERS ALL WORKING TOGETHER.

OTHER PLACES YOU HAVE NICE, AND HERE WE HAVE ICER. THERE'S ALL SORTS OF OTHER MODELS THAT

WE CAN LOOK AT IS MY COMMENT. AND, AGAIN, I'M HESITANT TO SAY ANYTHING HERE BECAUSE I'M REALLY NOT THE MOST INFORMED IN THIS AREA. BUT I'M JUST TRYING TO BRING MY EXPERIENCES FROM NONREGENERATIVE MEDICINE LESSONS. AND THINGS MOVE VERY SLOWLY WHEN YOU LEAVE IT IN THE HANDS OF THE PAYER.

VICE CHAIR BONNEVILLE: THANK YOU. ANYBODY HAVE ANY OTHER QUESTIONS ON THIS TOPIC?

WE HAVE ANOTHER PRESENTATION. I WANTED TO SEE IF THERE WAS ANY PUBLIC COMMENT SPECIFICALLY ON THIS TOPIC CURRENTLY. SO, MARIANNE, I DON'T KNOW IF YOU SEE ANY HANDS RAISED.

MS. DEQUINA-VILLABLANCA: I DON'T SEE ANY HANDS RAISED. IF ANYONE NEEDS A REMINDER ON HOW TO UNMUTE THEMSELVES, IT'S STAR NINE. BUT CURRENTLY, MARIA, I DO NOT SEE ANY HANDS RAISED.

VICE CHAIR BONNEVILLE: GREAT.

OUR NEXT PRESENTATION IS BY GEOFF LOMAX.

DR. LOMAX: GOOD AFTERNOON. AND THANKS VERY MUCH, MARIA.

I'M GOING GIVE YOU A BRIEF UPDATE ON OUR MOST RECENT LISTENING SESSION. AS YOU MAY RECALL, AT THE FEBRUARY MEETING, I UPDATED YOU ON THE COMMUNITY CARE CENTERS OF EXCELLENCE PROGRAM, WHICH SEAN ALLUDED TO IN THE PREVIOUS PRESENTATION. THIS

IS REALLY A PROGRAM MEANT TO EXTEND OUR CLINICAL REACH BEYOND THE ACADEMIC CENTERS AND INTO THE COMMUNITY, PARTICULARLY THOSE THAT HAVE BEEN LESS SERVED BY CLINICAL TRIALS AND SOME OF OUR CLINICAL PROGRAMS.

AND I DESCRIBED OUR CONVERSATION AT UC RIVERSIDE, WHICH IS PART OF OUR INLAND EMPIRE NEEDS ASSESSMENT. AND LAST WEEK, AND I'M JUST GIVING YOU A VERBAL BECAUSE WE JUST GOT BACK AND WE DIDN'T HAVE TIME TO MEET THE POSTING DEADLINE FOR SLIDES. BUT WE WILL HAVE SOME MATERIALS IN THE FUTURE AND CAN SEND THOSE OVER TO YOU.

LAST WEEK WE TRAVELED TO PALM DESERT WITH THE AIM OF ENGAGING COMMUNITY-BASED ORGANIZATIONS AND REGIONAL HEALTHCARE PROVIDERS. THAT WAS AN AUDIENCE WE WANTED TO MAKE SURE WE INCLUDED IN THIS FIRST PHASE OF THE NEEDS ASSESSMENT. AND THESE LISTENING SESSIONS ARE REALLY AIMED TO UNDERSTAND REGIONAL NEEDS AND CAPACITIES. AND SPECIFICALLY, WE ORGANIZE OUR CONVERSATION INTO SORT OF THREE AREAS: THE CLINICAL CAPACITY TO SUPPORT PATIENTS IN THEIR JOURNEY, TRAINING AND EDUCATION NEEDS AMONG HEALTHCARE PROVIDERS, EDUCATORS, OR NAVIGATORS, AND THE THIRD BEING MORE THE GENERAL INFORMATION NEEDS FOR PROVIDERS, PATIENTS, THE PUBLIC. SO KIND OF A

CLINICAL, A TRAINING, AND THEN A COMMUNITY ENGAGEMENT ASPECT IN TRYING TO UNDERSTAND WHAT WE CAN DO IN THOSE AREAS TO REALLY ADVANCE CIRM'S MISSION AND PROPOSITION 14'S MANDATED ROLE OF THE COMMUNITY CARE CENTERS.

AGAIN, I REPORTED ON THESE AREAS IN THE PAST, AND THERE'S SOME SLIDES THAT I THINK REALLY CAPTURE SOME OF THE THINKING. SO I'M GOING TO MAKE SOME BRIEF REMARKS ABOUT HOW THEY RELATE TO THE INLAND EMPIRE CONVERSATION WHICH, AGAIN, WAS A LITTLE BIT OF A DIFFERENT CONVERSATION IN THE SENSE THAT WE WERE ENGAGING WITH COMMUNITY-BASED ORGANIZATIONS AND THEN SOME ORGANIZATIONS WITH A UNIQUE FOOTPRINT IN THE INLAND EMPIRE.

ON THE CLINICAL SIDE, WE VISITED A CLINICAL RESEARCH CENTER OPERATED BY A REGIONAL PROVIDER SERVING ABOUT 65,000 PATIENTS IN THE REGION. PERHAPS THE HIGHEST VALUE OBSERVATION WE HAD FROM THE PERSPECTIVE OF CLINICAL TRIALS WAS THE PATIENTS COULD PARK WITHIN ABOUT A HUNDRED FEET OF THE CENTER AND ACCESS IT VERY EASILY AND EFFICIENTLY, WHICH COMPARED TO SOME OF OUR URBAN MEDICAL CENTERS, THAT'S A HUGE PLUS. THE KIND OF ACCESSIBILITY AND SORT OF COMMUNITY-FACING NATURE OF THESE CENTERS IS ACTUALLY VERY IMPRESSIVE. I FOUND

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IT QUITE STRIKING. AGAIN, VISITED ALL THE ALPHA CLINICS AND HALF THE TIME GETTING LOST TRYING TO ACTUALLY FIND THE PLACE I'M SUPPOSED TO MEET WITH FOLKS. SO THAT LOCAL TOUCH IS ACTUALLY, I THINK, GOING TO SERVE US WELL.

THE CLINICAL CENTER WAS ACTUALLY WELL VERSED IN CLINICAL TRIAL OPERATIONS AND, IN ADDITION, DID HAVE RELATIONSHIPS WITH MULTIPLE ALPHA CLINIC SITES. SO THE IDEA THAT THESE CENTERS CAN WORK WITH OUR EXISTING NETWORKS SEEMS LIKE A VERY VALID HYPOTHESIS THAT WE CAN DEVELOP THROUGH THIS PROGRAM.

CELL AND GENE THERAPY WAS NOT AN AREA OF EXPERIENCE OR EXPERTISE; BUT IF WE ASSUME A PATIENT MIGHT STILL RECEIVE TREATMENT, SAY, IN A MAJOR CENTER AND THAT THERE ARE CERTAIN PRE- AND POST-TREATMENT ACTIVITIES THAT COULD GO IN THE LOCAL AREA, ONE COULD REASONABLY ANTICIPATE THAT THESE CENTERS COULD THEN SUPPORT THE PROGRAMS BASED ON THAT HYPOTHESIS. SO ON THE CLINICAL SIDE, IT WAS, LIKE I SAY, WE LEARNED A LOT AND SAW A LOT THAT WAS VERY PROMISING.

ON TRAINING AND EDUCATION, I THINK THE MESSAGE THAT'S REALLY BUILDING, KIND OF THE WEIGHT OF EVIDENCE THAT'S BUILDING THROUGH THESE

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CONVERSATIONS IS THAT THERE IS AN OPPORTUNITY TO LEVERAGE EXISTING CAPACITIES. THOSE CAPACITIES, SOME OF THEM MIGHT BE HOMEGROWN CIRM CAPACITIES; FOR EXAMPLE, THE BRIDGES PROGRAM. THERE'S A SAN BERNARDINO PROGRAM OUT THERE AND THEY'RE QUITE ACTIVE. SO SOME OF OUR EXISTING EDUCATION PROGRAMS CAN REALLY BE BROUGHT IN AND LEVERAGED HERE.

BUT IN ADDITION, I THINK ONE EXAMPLE THAT REALLY STOOD OUT IN THIS CONVERSATION WAS THE POTENTIAL FOR THE PROMOTORES THAT REALLY PROVIDES A HIGH LEVEL, A HIGH DEGREE OF MEDICALLY RELEVANT NAVIGATION GUIDANCE AND INFORMATION TO COMMUNITIES THAT REALLY HAVEN'T TRADITIONALLY, I THINK, BEEN REPRESENTED IN CIRM PROGRAMS, PARTICULARLY CLINICAL PROGRAMS. AND IT MAY NOT BE EVEN -- I THINK WE COULD LOOK AT IT FROM THE STANDPOINT OF, EVEN IF THE PROMOTORES AREN'T -- THE END GAME ISN'T THEY'RE GOING TO GET PATIENTS INTO CLINICAL TRIALS. THERE'S A LOT OF VALUE THEY CAN BRING.

SO, FOR EXAMPLE, WE HAD A LOT OF INTERESTING DISCUSSION ABOUT THE FACT THAT HEALTHCARE DELIVERY IN THAT REGION INCLUDES MEDICAL TOURISM FOR VERY LEGITIMATE PURPOSES BECAUSE OF COST. SO THAT YOU HAVE INDIVIDUALS MOVING BACK AND FORTH IN THE BORDER REGION, AND PROMOTORES PROVIDE A

VERY IMPORTANT ROLE IN TERMS OF HELPING NAVIGATE SOME OF THOSE PATIENTS.

IN ADDITION, THEY HAVE A CERTIFICATION PROGRAM NOW. SO ONE OF THE THOUGHTS WAS TO WHAT EXTENT COULD THIS INITIATIVE HELP INFORM THOSE CERTIFICATION PROGRAMS. SO CERTAINLY THEY'RE ABLE TO DISTINGUISH BETWEEN, SAY, WHAT DOES A TESTED AND WELL-EVALUATED TREATMENT LOOK LIKE VERSUS A COMPLETELY UNREGULATED TREATMENT AND GIVING PATIENTS TOOLS TO MAKE THOSE DISTINCTIONS.

THOSE WERE SOME OF THE IDEAS, SOME OF THE THINGS THAT CAME UP IN THE TRAINING AND EDUCATION PIECE.

AND THEN FINALLY, ON THE PUBLIC-FACING COMMUNICATIONS, I THINK IT'S REALLY IMPORTANT THAT, AND WE GOT THIS MESSAGE COMING ACROSS, AND THE COMMUNICATIONS TEAM HAS NOW BECOME VERY INVOLVED WITH THESE SESSIONS, IS THAT REALLY LOOKING -- CIRM CAN DEVELOP TOOLS AND COMMUNICATIONS TOOLS THAT THEN REALLY THE ONLY WAY THEY'RE GOING TO HAVE AN IMPACT, WE SEE, IS REALLY BY DISSEMINATING THOSE TOOLS INTO, AGAIN, BOTH OUR EXISTING PROGRAMS, BUT PERHAPS LIKE THESE PROMOTORES PROGRAMS, GOING BACK TO THE CLINIC, LIKE WORKING WITH THE CLINICS TO HAVE CUSTOMIZABLE EDUCATION TOOLS THAT ARE A FIT FOR THEIR PARTICULAR

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AUDIENCE. SO HAVING SOME CAPACITY TO START WITH WELL-DEVELOPED CIRM INFORMATION TOOLS THAT COME OUT OF THE CIRM SHOP AND THEN DISSEMINATE THROUGH OUR NETWORKS IN A WAY THAT STILL ALLOWS FOR FURTHER CUSTOMIZATION, AGAIN, BY A COMMUNITY CARE CENTER, BY A PROGRAM AWARDED THROUGH THAT CENTER.

SO, AGAIN, A LOT OF GOOD EXAMPLES, A LOT OF RICH DISCUSSION CAME OUT OF THAT. WITH THAT, I'LL STOP AND TURN IT BACK TO THE COMMITTEE.

VICE CHAIR BONNEVILLE: I WANTED TO JUST MENTION THE TEAM'S DONE EXTRAORDINARY WORK ON THESE LISTENING SESSIONS. THERE HAVE BEEN THREE. THERE WILL BE ANOTHER ONE IN MAY IN SACRAMENTO WHICH WILL BE OPEN TO THE PUBLIC AND OPEN TO ANY OF THE MEMBERS OF THIS COMMITTEE THAT WOULD LIKE TO ATTEND AS WELL AS BOARD MEMBERS.

SOMETHING WE'VE HEARD OVER AND OVER AGAIN, AND I KNOW GEOFF WILL AGREE WITH THIS, IS JUST THE WHOLE CONCEPT OF TRUST AND HOW WE BUILD THAT IN THESE COMMUNITIES. AND I KNOW THAT THAT'S NOT A SHOCK TO ANYONE. IT'S ALSO, THOUGH, TRUST IN US AS AN ORGANIZATION. SO THEY DON'T KNOW US. THEY DON'T KNOW THE WORK WE FUND. SO BUILDING THAT TRUST FOR CIRM IS ALSO REALLY IMPORTANT AND WE HEARD THAT.

SO I JUST REALLY WANT TO THANK THE MED

AFFAIRS FOR ALL THE HARD WORK THEY'VE DONE AT THESE VARIOUS LISTENING SESSIONS. AND THEY'VE ALL BEEN GREAT TO ATTEND, AND THE INFORMATION WE'VE GOTTEN HAS BEEN REALLY WONDERFUL.

J.T.

CHAIRMAN THOMAS: I'D LIKE TO ECHO THAT AS WELL. THEY'VE BEEN EXCELLENT, AND THE THREE HAVE ALL BEEN SIMILAR IN CERTAIN RESPECTS AND DIFFERENT. IT'S A FUNCTION OF GEOGRAPHY AND PATIENT GROUP, ET CETERA.

I WANTED TO ADD, I WAS GOING TO COMMENT ON THE TRUST POINT AS WELL, BUT THE OTHER CHALLENGE THAT WE HAVE IS THE FIELD ITSELF IS ONE THAT IS NOT AT ALL WELL UNDERSTOOD BY THE POTENTIAL PATIENT BASE OR MANY OTHER PEOPLE, FOR THAT MATTER, BECAUSE IT'S NEW AND IT'S EVOLVING, ET CETERA. SO A MAJOR CHALLENGE, GETTING BACK TO THE EDUCATIONAL COMPONENT, IS TO EDUCATE THE MEMBERS OF THE PUBLIC IN ANY GIVEN AREA THAT WE ARE LOOKING TO GO INTO ON WHAT THE FIELD OF REGENERATIVE MEDICINE IS ALL ABOUT, WHICH IS NOT AN EASY LIFT. BUT IF YOU CAN'T DO THAT, THAT THEN FEEDS FURTHER INTO THE MISTRUST ELEMENT BECAUSE THE POTENTIAL PATIENTS IN THE PROGRAMS OR THE TRIALS OR WHATEVER ARE NOT GOING TO KNOW SORT OF WHAT IT IS THEY'RE ACTUALLY GETTING

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INTO. SO IT'S DIFFICULT TO UNDERSTATE THE IMPORTANCE OF THE EDUCATIONAL COMPONENT OF ALL THIS.

AND I THINK THAT WE ARE DOING A VERY GOOD JOB OF PUTTING TOGETHER THINGS FOR THE PUBLIC, BUT IT'S SOMETHING WE NEED TO DO EVEN BETTER BECAUSE IT'S TRICKY TO COMPREHEND.

BUT TO GO FULL CIRCLE, THOUGH, I JUST DO WANT TO SAY THAT THE MED AFFAIRS TEAM HAS DONE JUST AN EXCELLENT JOB. AND THAT SESSION IN MAY, FOR THOSE OF YOU WHO ARE IN NORTHERN CALIFORNIA, WOULD BE, IF YOU HAVE AN OPPORTUNITY TO COME TO THAT, IT WOULD BE WELL WORTH YOUR TIME.

DR. LOMAX: THE MAY SESSION WILL BE HYBRID AS WELL, BUT WE'D LOVE TO HAVE YOU IN SACRAMENTO.

JUST ONE OTHER, I THINK IT'S A NICE STORY TO TELL BECAUSE IT REALLY SHOWS HOW ALL THE CIRM WORK GROUPS, HOW WE'RE CROSS POLLINATING THESE EFFORTS.

I JUST WANT TO, DR. LEVINE, ACKNOWLEDGE WHEN WE HAD THIS DISCUSSION AT THE LAST MEETING, YOU MADE SOME VERY POIGNANT STATEMENTS ABOUT SORT OF WHAT THE CLINICAL TRIALS WE ARE FUNDING REALLY MEANS TO PATIENTS. IT'S KIND OF LIKE AHA. IT WAS JUST THE WAY YOU KIND OF FRAMED THAT. WE ACTUALLY SORT OF WORKED THAT INTO THE CONVERSATION. I THINK IT

WAS A VERY IMPORTANT POINT, THAT THESE AREN'T JUST SORT OF RANDOM MEDICAL EXPERIMENTS. IN MOST CASES THEY'RE THE BEST OPTION THE PATIENT HAS. AND THAT ARTICULATION WAS QUITE VALUABLE, AND SO YOUR FEEDBACK DOES KIND OF GO BACK INTO THESE SESSIONS AND VICE VERSA. SO APPRECIATED THAT ONE.

DR. LEVINE: THANKS.

DR. TURBEVILLE: I THINK WE MAY HAVE LOST BONNEVILLE THERE. YEAH, MAYBE WE DID. OKAY. I WANT TO THANK EVERYBODY FOR THEIR CONSIDERATION. I KNOW WE ARE MOVING RAPIDLY FOR THE ROADMAP TO ACCESS AND AFFORDABILITY. AGAIN, I MENTIONED THE NEXT TOPIC, WHICH IS VERY INTERESTING, ON THE COVERAGE ANALYSIS, ACCESS AND APPEALS SPECIFICALLY FOR CELL AND GENE THERAPY.

WITH THAT, LET ME OPEN IT UP TO ANY FINAL COMMENTS OR QUESTIONS. I THINK BONNEVILLE MAY HAVE DROPPED OFF.

MR. TOCHER: SHE SHOULD BE COMING BACK ON. SHE JUST LOST HER POWER BUT IT'S RESUMING.

MS. DEQUINA-VILLABLANCA: CURRENTLY I DON'T SEE ANY HANDS RAISED OR QUESTIONS.

DR. TURBEVILLE: OKAY. VERY GOOD. I DO HAVE SOME FOLLOW-UP. I'LL SEND OUT SOME INFORMATION FOR THE REQUEST PARTICULARLY ON POST-MARKETING, THE

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DUE DILIGENCE THAT WE HAVE FOR OTHER CELL AND GENE THERAPIES THAT ARE NOW COMMERCIALLY APPROVED. I THINK THAT WOULD PROVIDE SOME GOOD INSIGHT. AND LOOK FORWARD TO THE NEXT PRESENTATION ON ACCESS AND AFFORDABILITY. WITH THAT, GO AHEAD AND CLOSE US DOWN UNLESS, J.T., YOU HAVE ANY.

CHAIRMAN THOMAS: I WOULD JUST GIVE MARIA A SECOND TO GET BACK ON HERE.

MR. TOCHER: SHE'S HAVING DIFFICULTY. I THINK WE CAN WRAP IT UP.

MS. DEQUINA-VILLABLANCA: WE DO NEED TO CALL FOR PUBLIC COMMENT, WHICH I DON'T SEE ANY CURRENTLY. SO I THINK WE ARE GOOD.

DR. TURBEVILLE: OKAY. WITH THAT, I APPRECIATE EVERYBODY'S TIME. I KNOW YOU'RE TERRIBLY BUSY, AND I LOOK FORWARD TO THE NEXT AAWG.

CHAIRMAN THOMAS: SEAN, LET ME JUST ADD ONE LAST THING, IF I MAY. SO FOR THOSE OF YOU ON THE AAWG OUTSIDE OF CIRM, YOU MAY KNOW WE ELECTED A NEW CHAIR ON JANUARY 26TH. HE IS DR. VITO IMBASCIANI, CURRENTLY THE SECRETARY OF VETERANS AFFAIRS IN GOVERNOR NEWSOM'S ADMINISTRATION. HE WILL BE SWORN IN AT OUR MARCH 28TH BOARD MEETING. AND AS A RESULT, THAT MAKES THIS MY LAST AAWG AS CHAIR OF CIRM. AND I JUST WANTED TO THANK ALL OF

YOU FOR YOUR PARTICIPATION AND YOUR GREAT INSIGHTS AND HELP IN THIS CRITICAL AREA GOING FORWARD, WHICH IS A VERY DIFFICULT ONE. BUT ALL OF YOUR THOUGHTS AND COUNSEL GREATLY AIDS CIRM IN BEING ABLE TO ADVANCE THE BALL HERE UNDER THE MEDICAL AFFAIRS TEAM. SO THANK YOU, EVERYBODY. AND I KNOW THINGS WILL ONLY GET BIGGER AND BETTER AS WE GO ON, AND IT'S BEEN A PLEASURE WORKING WITH ALL OF YOU. AND I WILL BE VIEWING FROM AFAR TO ROOT YOU GUYS ON. SO THANKS VERY MUCH.

(THE MEETING WAS THEN CONCLUDED AT 2:04 P.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE VIRTUAL PROCEEDINGS BEFORE THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON MARCH 14, 2023, 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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