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: JANUARY 13, 2023
2:30 P.M.

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

FILE NO.: 2023-02

I N D E X

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FRIDAY, JANUARY 13, 2023; 2:30 P.M.

MS. DEQUINA-VILLABLANCA: I WILL START WITH THE ROLL CALL.

DAN BERNAL. ANN BOYNTON.

DR. BOYNTON: HERE.

MS. DEQUINA-VILLABLANCA: JAMES DEBENEDETTI.

MR. DEBENEDETTI: HERE.

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

DR. HIGGINS: HERE.

MS. DEQUINA-VILLABLANCA: PAT LEVITT.

DR. LEVITT: HERE.

MS. DEQUINA-VILLABLANCA: EXCUSE ME. I SKIPPED A NAME. HARLAN LEVINE. APOLOGIES.

DR. LEVINE: I'M HERE.

MS. DEQUINA-VILLABLANCA: ADRIANA PADILLA. AMMAR QADAN.

DR. QADAN: HERE.

MS. DEQUINA-VILLABLANCA: AL ROWLETT. DAVID SERRANO-SEWELL. MAHESWARI SENTHIL.

DR. SENTHIL: HERE.

MS. DEQUINA-VILLABLANCA: ADRIENNE SHAPIRO.

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MS. SHAPIRO: HERE.

MS. DEQUINA-VILLABLANCA: JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MR. BERNAL: DAN BERNAL IS HERE.

MS. DEQUINA-VILLABLANCA: ART TORRES.

CHAIRMAN TORRES: PRESENT.

MS. DEQUINA-VILLABLANCA: AND THEN WE'VE GOT DAN BERNAL PRESENT. OKAY.

CHAIRMAN TORRES: HOW CLOSE ARE WE TO A QUORUM?

MS. DEQUINA-VILLABLANCA: WE ARE AT ONE SHORT OF QUORUM.

CHAIRMAN TORRES: OKAY. WELL, WITH THE PERMISSION OF THE GROUP, I INTEND TO PROCEED SINCE WE ARE NOT GOING TO BE TAKING ANY VOTES DURING THIS MEETING. I THINK WE ARE NOT GOING TO NEED A QUORUM, BUT IT'S JUST GOOD TO HAVE ONE JUST TO CONDUCT BUSINESS.

THIS IS MY LAST MEETING AS YOUR CHAIR. I AM TERMED OUT AFTER 13 YEARS OF SERVICE TO CIRM ON JANUARY 26TH ALONG WITH MY ESTEEMED CHAIRMAN, JONATHAN THOMAS, WHOM I HAVE ENJOYED SERVING WITH THROUGHOUT THAT PERIOD OF TIME. IT'S BEEN QUITE AN INSPIRING EXPERIENCE, AND ALSO, OF COURSE, SERVING WITH DR. MILLAN AND MARIA BONNEVILLE AND OTHERS WHO

ARE ON THIS PAGE, INCLUDING SCOTT AND GEOFF LOMAX. I JUST WANT TO THANK YOU ALL FOR YOUR SERVICE.

I'M VERY MINDFUL THAT IT'S A FRIDAY AFTERNOON. SO WE'RE GOING TO PROCEED AHEAD AND GO INTO OUR REPORTS. I'M GOING TO SHIFT IT NOW TO SEAN TURBEVILLE, WHO WILL BE INTRODUCING OUR SPEAKERS, WHICH I THINK IS GOING TO BE A VERY INFORMATIVE PRESENTATION. THANK YOU, SEAN.

DR. TURBEVILLE: ALL RIGHT. THANK YOU, VICE CHAIRMAN, MR. CHAIRMAN, EVERYBODY. GOOD AFTERNOON. THERE'S TWO PRESENTATIONS TODAY. I'M GOING TO PUNT IT OVER TO OUR VICE PRESIDENT OF CLINICAL, ABLA, WHO'S GOING TO GIVE A PORTFOLIO OVERVIEW. AND THEN AFTER THAT I WILL PRESENT AN UPDATE FROM THE AAWG.

CHAIRMAN TORRES: DR. CREASEY.

DR. CREASEY: HERE WE GO.

CHAIRMAN TORRES: START OVER.

DR. CREASEY: CAN YOU SEE MY SLIDES?

CHAIRMAN TORRES: WE SEE YOUR SLIDES. WE JUST COULD NOT HEAR YOU BECAUSE YOU WERE ON MUTE.

DR. CREASEY: OKAY. THANK YOU. WELL, GOOD AFTERNOON, EVERYONE. MY NAME IS ABLA CREASEY. I'M IN THERAPEUTICS DEVELOPMENT AT CIRM. I'M GOING GIVE YOU TODAY A BRIEF PUBLIC OVERVIEW OF OUR ACTIVE

CLINICAL TRIAL PORTFOLIO. IT COVERS THE CLINICAL TRIALS GRANTS THAT ARE CURRENTLY IN OUR ACTIVE INVOLVEMENT AND HAVE WORKED IN COLLABORATION, CLOSE COLLABORATION, WITH THE FDA AND HAD APPLIED TO CIRM FOR FUNDING.

MY GOAL TODAY REALLY IS NOT TO JUST GIVE YOU A DOWNPOUR OF WHAT WE DO, BUT REALLY TO FAMILIARIZE YOU WITH OUR CLINICAL PORTFOLIO IN A PUBLIC FORUM SO YOU HAVE A FEEL FOR HOW IT LOOKS LIKE.

SO WE ALWAYS START OUR PRESENTATION WITH THE CIRM MISSION, ACCELERATING WORLD-CLASS SCIENCE TO DELIVER TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND WORLD.

SO I'M GOING TO CONCENTRATE ON THE MIDDLE PART OF THE SLIDE, WHICH IS ADVANCING THERAPIES TO MARKETING APPROVAL UNDER DELIVER REAL-WORLD SOLUTIONS. MY TEAM AND I, THAT IS, THE THERAPEUTICS DEVELOPMENT TEAM, MANAGE TRANSLATION AND CLINICAL GRANTS. CIRM HAS BEEN SUCCESSFUL IN ATTRACTING AND DERISKING PROJECTS IN EARLY STAGE DEVELOPMENT, PREPARING THEM FOR A PRE-IND MEETING OR CONDUCTING IND-ENABLING STUDIES AND THEN PROGRESSING TO EARLY CLINICAL DEVELOPMENT. SOME ARE PROGRESSING TO MID

TO LATE CLINICAL DEVELOPMENT, AND I WILL COVER LATER IN THE PRESENTATION WHICH OF THOSE THAT ARE LATER IN THE DEVELOPMENT.

AS YOU SEE, MUCH OF THE PORTFOLIO IS CURRENTLY IN EARLY CLINICAL DEVELOPMENT. AND THE TRANSLATION CLINICAL GRANTS ARE CONCENTRATED IN THREE MAIN THERAPEUTIC AREAS: NEUROLOGY, THE GREEN PART; ONCOLOGY, AND THAT COVERS THE BLOOD AND SOLID CANCERS; HEMATOLOGICAL DISORDERS, SUCH AS SICKLE CELL AND THALASSEMIA.

MY TEAM AND I REACH OUT TO ACADEMICS AND FOR-PROFIT ORGANIZATIONS AND ENCOURAGE THEM TO APPLY AND FREQUENTLY ADVISE THEM AND CONSULT FOR THEM IN GRANT PROPOSAL ASSEMBLY.

THE GRADUAL GROWTH OF THE CLINICAL PORTFOLIO IS SHOWN ON THIS SLIDE. WE WERE ABLE TO ATTRACT AND FUND 86 CLINICAL GRANTS IN SEVERAL THERAPEUTIC AREAS BY THE END OF 2022. OVER 50 PERCENT OF OUR FUNDED CLINICAL TRIALS ARE PARTNERED WITH INDUSTRY.

I'M GOING TO GO THROUGH THE NEXT FOUR SLIDES QUICKLY TO JUST -- AGAIN, YOU CAN ACTUALLY VIEW THAT KIND OF DATA ON OUR WEBSITE. I JUST WANTED TO MAKE A COUPLE OF POINTS.

THERE ARE CURRENTLY 30 ACTIVE PHASE 1

TRIALS. FIFTEEN OF THEM ARE SHOWN ON THIS SLIDE. PATIENT NUMBER, TARGETED ENROLLMENT, PATIENT NUMBER IS THE LAST COLUMN. SO KEEP AN EYE ON THAT. HERE'S THE NEXT 15 PHASE 1 TRIALS. MOST OF THE TRIALS TEND TO INCLUDE A RELATIVELY SMALL NUMBER OF PATIENTS AND ARE IN DIVERSE THERAPEUTIC AREAS AS SEEN BY THE PIE CHART AND ALSO DEMONSTRATING WITH THESE TABLES.

THERE ARE CURRENTLY 12 PHASE 1-2 ACTIVE TRIALS PER AGREEMENT WITH THE FDA. THESE TRIALS ARE DESIGNED TO ASSESS SAFETY AND EARLY OR PRELIMINARY EVIDENCE OF EFFICACY. FOUR OF THE CLINICAL TRIALS ARE IN PHASE 2, TWO IN OPHTHALMOLOGY, ONE IN INFECTIOUS DISEASE, AND ONE IN SICKLE CELL ANEMIA. IF I'M HURRYING, PLEASE STOP ME BECAUSE I WOULD LIKE YOU TO ABSORB SOME OF THIS INFORMATION.

ALL ARE IN DIFFERENT ADVANCEMENT STAGES. SEVERAL OF OUR GRANTS PROGRESS FROM DISCOVERY TO TRANSLATION TO CLIN1 AND CLIN2. I'M JUST GOING TO GIVE YOU AN EXAMPLE LIKE WHY DO WE HAVE TWO OPHTHALMOLOGY GRANTS FOR DR. HENRY KLASSEN. YOU CAN SEE HERE HE'S HAD HIS PROGRAM WITH US SINCE DISCOVERY, TRANSLATION, AND ALSO NOW HAS HAD TWO CLINICAL GRANTS TO ADVANCE THE PRODUCT. THEY COME IN AT DIFFERENT TIMES AND THEY HAPPEN TO OVERLAP IN TERMS OF ACTIVITY.

ADVANCING THE PORTFOLIO IS A DYNAMIC PROCESS THAT ENGAGES THE GRANTEES, CIRM, ALMOST ALL THE PARTS OF CIRM, PATIENT REPRESENTATIVES, AS WELL AS EXTERNAL EXPERTS. WE DO THAT THROUGH CLINICAL ADVISORY PANELS THAT WE DEARLY CALL CAP'S. THE PURPOSE OF THE CAP IS TO PROVIDE GUIDANCE AND ADVICE TO THE PROJECT TEAM. THE CAP IS ASSEMBLED BY CIRM FOR EACH CLINICAL STAGE AWARD. THAT PROCESS STARTED BEFORE I JOINED CIRM IN 2016, BUT WE EXECUTED ON IT STARTING 2016. MULTIPLE CAP MEETINGS OCCUR OVER A LIFETIME OF A PROGRAM AWARD. WE FOLLOW EACH OF THE PROGRAMS CLOSELY.

YOU CAN TELL THAT THE NUMBERS OR STATISTICS LOOK RATHER IMPRESSIVE ON THE SLIDE WITH THE NUMBER OF CAP'S, HOW MANY ADVISORS, THE PATIENT REPRESENTATIVES. THE QUESTION THAT I'M SURE YOU'RE ASKING: DO THESE CAP'S MAKE ANY DIFFERENCE? THE ANSWER IS YES AS MEASURED BY WHETHER THE CHALLENGES THOSE GRANTEES ENCOUNTER WERE RESOLVED OR NOT. DID THEY RECEIVE THE REGULATORY DESIGNATION THEY WANTED? IN GENERAL, THE CLINICAL ADVISORY PANELS HAVE BEEN SUCCESSFUL IN ASSISTING THE GRANTEES IN OVERCOMING WHAT YOU SEE HERE ON THE OUTCOMES: MANUFACTURING CHALLENGES, CLINICAL TRIAL DESIGN OPTIMIZATION, ENROLLMENT ENHANCEMENT, REGULATORY ADVICE PROVIDED

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BEYOND WHAT THEY GET FROM THE FDA AND OTHERWISE, PARTNERING FACILITATED, AND THE DEVELOPMENT PATH ESSENTIALLY DELINEATED WITH OUR HELP.

SO NOW I'M GOING TO GIVE YOU A BRIEF DESCRIPTION OF THE CURRENT MOST ADVANCED CLINICAL TRIALS IN OUR PORTFOLIO THAT HAVE HAD CLOSE INTERACTIONS WITH THE FDA AND SOME OF THEM EVEN WITH THE AMEA.

SO I DON'T REPEAT MYSELF, SIX OUT OF THE EIGHT GRANTS THAT I'M GOING TO SHARE WITH YOU HAVE RECEIVED AN ACCELERATED DESIGNATION FROM THE FDA, WHETHER IT IS THE RMAT, WHICH IS REGENERATIVE MEDICINE ADVANCED THERAPY, OR BREAKTHROUGH. AND THAT'S WHAT I CALL CLOSE INTERACTIONS WITH THE REGULATORS, AND WITH OTHERS WE KNOW THEY HAVE ONGOING CONVERSATIONS THAT THEY HAVE NOT YET BEEN AWARDED THE ACCELERATED APPROVAL DESIGNATION.

SO WE'RE GOING TO START WITH A CELL THERAPY FOR RETINITIS PIGMENTOSA. THIS PROGRAM IS PIONEERED BY DR. HENRY KLASSEN AT UC IRVINE. DR. KLASSEN RECEIVED SEVERAL GRANTS FROM US, AS I POINTED OUT EARLIER. THE PHASE 2 STUDY IDENTIFIED A BIOMARKER THAT IS GOING TO BE HELPFUL IN THE DESIGN OF THE FOLLOW-UP OF A PHASE 3 STUDY.

ON THIS SLIDE YOU SEE ONE OF HIS PATIENTS,

ROSIE BARRERO, WHO'S ONE OF THE FIRST PATIENTS WHO RECEIVED ONE INJECTION OF THIS THERAPY. ROSIE CAN NOW SEE HER KIDS -- SHE WASN'T ABLE TO SEE HER CHILDREN -- DRIVE A CAR, AND SHE LIVES A NORMAL LIFE.

GENE THERAPY FOR PATIENTS WHO LACK THE ENZYME ADENOSINE DEAMINASE AND END UP WITH A SEVERE COMBINED IMMUNODEFICIENCY SYNDROME, WHICH WE ALSO USE THE ACRONYM, SCID BUBBLE BABY DISEASE, IS A PROGRAM THAT'S PIONEERED BY DR. DON KOHN OF UCLA. DON ENROLLED 50 PATIENTS WITH ADA-SCID, 30 IN THE U.S. AND 20 IN THE UK. DATA FROM THE TWO U.S. STUDIES THAT HE'S DONE, 24 MONTHS OF FOLLOW-UP WERE ANALYZED ALONGSIDE DATA FROM THE UK STUDY THAT WAS FOLLOWED UP TO 36 MONTHS SHOWED THAT THE CHILDREN ARE DOING VERY WELL AND THAT THE TREATMENT LED TO RESOLUTION OF THE DISEASE FOR MOST PATIENTS, MOST CHILDREN.

AND HERE YOU SEE ONE OF THE U.S. PATIENTS, EVIE, WHO WAS TREATED AT AGE TWO. SHE'S JUMPING HAPPILY AND ALSO LIVING A NORMAL LIFE. THE RESUMPTION OF THIS CLINICAL TRIAL BY DR. KOHN IS IN PROGRESS. HE'S BEEN WAITING, BUT A RECENT GO AHEAD BY THE FDA. RECRUITMENT FOR THE TRIAL STARTED WITH ENROLLMENT BEGINNING VERY SOON.

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THE NEXT TRIAL IS GENE THERAPY FOR PATIENTS WHO LACK A GENE CALLED CD 18 THAT LEADS TO, AGAIN, IMMUNE DYSFUNCTION. AND THE CHILDREN SUFFER CHRONIC BACTERIAL AND VIRAL INFECTIONS. DR. PATEL OF ROCKET PHARMA IS THE PRINCIPAL INVESTIGATOR WITH THIS GRANT. THEY'VE TREATED NINE PATIENTS AND ALL ARE DOING WELL WITH A DEMONSTRATED BENEFIT AND SURVIVAL, DECREASED HOSPITALIZATION, AND RESOLUTION OF VARIOUS OTHER DISEASE-RELATED ISSUES SUCH AS SKIN. AGAIN, THEIR SKIN GOT BETTER, NO RASHES.

HERE IS ONE OF THE PATIENTS, MARLEY, WHO IS NOW LIVING ALSO A NORMAL LIFE WITH MINIMAL COMPLICATIONS. ROCKET PHARMA HAS INITIATED DISCUSSIONS WITH THE REGULATORS REGARDING FILING PLANS FOR THEIR PRESS RELEASE.

CELL THERAPY FOR KIDNEY TRANSPLANTS IS ANOTHER EXCITING PROJECT IS A GRANT AWARDED TO MEDEOR, A CALIFORNIA BIOTECH COMPANY THAT WAS STARTED BY THE LATE DR. SAM STROBER OF STANFORD. DR. BRENNAN IS THE PI OF THIS GRANT. THE GOAL OF THIS KIDNEY TRANSPLANT PROJECT IS TO INDUCE TOLERANCE OF A KIDNEY TRANSPLANT BY GIVING THE ORGAN RECIPIENT HEMATOPOIETIC STEM CELLS FROM THE DONOR. THE ORGAN IS PROTECTED BY STANDARD IMMUNOSUPPRESSION, WHICH IS REALLY STANDARD

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PROTOCOL, FOR APPROXIMATELY SIX MONTHS. AND THIS IMMUNOSUPPRESSION ALSO PROTECTS THE PROVIDED DONOR CELLS THAT ARE HEMATOPOIETIC STEM CELLS, WHICH WE CALL THE GRAFT. THE DONOR T-CELLS PROVIDE TRANSIENT CHIMERISM WHICH BECOMES LONG TERM IF THE PROCEDURE IS SUCCESSFUL. WHEN THE HEMATOPOIETIC STEM CELLS ENGRAFT FOR ESSENTIALLY EVER, THE PATIENT GETS IMMUNOSUPPRESSION -- HE GETS OFF OF IMMUNE SUPPRESSION DRUGS.

THE STUDY DID A PLACEBO CONTROL AND TREATED PATIENTS, 20 TREATED AND 10 CONTROLS. THE PATIENTS ARE TO BE FOLLOWED FOR TWO YEARS. THAT'S THE STAGE BY WHICH THIS GRANT IS AT, THE STAGE OF FOLLOW-UP OF ALL THOSE 30 PATIENTS.

THIS NEXT GRANT DEALS WITH A MONOCLONAL ANTIBODY THAT DEPLETES BLOOD STEM CELLS AND ENABLES CHEMOTHERAPY-FREE TRANSPLANTS IN PATIENTS WITH SEVERE COMBINED IMMUNODEFICIENCY. THIS IS BEING DEVELOPED BY DR. WANG AND COLLEAGUES OF JASPER THERAPEUTICS.

PATIENTS WITH THE BUBBLE BOY DISEASE, THAT'S THE WAY THEY DESCRIBE IT, WHICH IS, AGAIN, ANOTHER SCID GRANT, CANNOT FIGHT INFECTIONS BECAUSE THEIR STEM CELLS DO NOT MAKE IMMUNE CELLS, IMMUNE T-CELLS AND B-CELLS.

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THE HUMANIZED MONOCLONAL ANTIBODY RESULTS IN A NICHE SPACE IN THE BONE MARROW WHICH GETS REPOPULATED, INTERESTINGLY, BY THE TRANSPLANT OF NORMAL DONOR STEM CELLS, RESULTING IN NORMAL LEVELS OF T AND B LYMPHOCYTES THAT CURE THEM OF THEIR DISEASE. USING THIS ANTIBODY LED TO ACTUALLY DONOR ENGRAFTMENT, NAIVE LYMPHOCYTE PRODUCTION, AND CLINICAL BENEFIT IN PATIENTS WITH THE BUBBLE DISEASE AND OF DIFFERENT MOLECULAR DEFICIENCIES. SO THEY'RE NOT DEFICIENT BECAUSE OF ONE ANTIGEN OR ANOTHER. THEY TRY MULTIPLE. SEVERAL PATIENTS HAVE BEEN TREATED TO DATE.

THIS GRANT THAT YOU SEE ON YOUR SLIDE IS THE ENDOTHELIAL -- ADMINISTRATION OF ENDOTHELIAL CELLS. THIS IS REPLETIVE MEASURE WHICH IS A VERY INTERESTING NOTION AS A THERAPEUTIC TO LYMPHOMA PATIENTS UNDERGOING HIGH-DOSE CHEMOTHERAPY AND AUTOLOGOUS HEMATOPOIETIC CELL TRANSPLANTATION. THIS PROGRAM IS LED DR. PAUL FINNEGAN OF ANGIOCRINE.

THE ENDOTHELIAL CELLS ARE GIVEN TO REDUCE THE DAMAGE CAUSED BY THE CHEMOTHERAPY TO THE HEALTHY BYSTANDER TISSUES AND REDUCE SEVERE REGIMEN-RELATED TOXICITIES WHICH ARE SERIOUS AND AT TIMES LIFE-THREATENING MORBIDITIES.

MANAGING THE CHEMOTHERAPY GREATLY IMPACTS

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THE QUALITY OF LIFE OF THE PATIENTS, BOTH THE SHORT AND LONG-TERM. THE GRANT COMPLETED A PHASE 1-2 STUDY THAT SHOWED PRELIMINARY SAFETY AND EFFICACY IN THIS APPROACH. THAT'S THE RATIONALE FOR WHY THEY GOT THE RMAT DESIGNATION, AS I TOLD YOU. AND THE STUDY NOW IS IN A PHASE 3 WHICH WAS RECENTLY SUBMITTED TO CIRM AND WAS RECENTLY APPROVED BY THE ICOC, AND THE TRIAL IS ALREADY ON ITS WAY.

CELL THERAPY FOR SPINA BIFIDA, WHICH IS BEING DEVELOPED BY DR. DIANA FARMER OF UC DAVIS. THE TREATMENT OF THE BABY TAKES PLACE WHEN THE BABY IS IN THE WOMB. DR. FARMER HAS PIONEERED DOING WORK IN THIS AREA. SHE HAS PUBLISHED LIKE IN 2004, I THINK, THE TRIAL, THE MOMS TRIAL, WHERE SHE SHOWED -- I'M SORRY -- THE ACTUAL MEMBRANE PATCH, BUT SHE HAS COME UP WITH A HYPOTHESIS THAT THEY GET BETTER TREATMENT WITH THE PATCH IN ADDITION TO THE CELLS. SO THE TEAM IS USING UNBANKED PLACENTAL MESENCHYMAL CELLS OF AN FDA-APPROVED AMNIOTIC MEMBRANE PATCH AS A TREATMENT. THE RESULTS ARE PROMISING WITH THREE INFANTS TREATED THUS FAR. THIS PILOT TRIAL IS IN PROGRESS AND IS ENROLLING WELL, AIMING FOR A TOTAL OF SIX PATIENTS.

HERE'S BABY TOBY WITH HIS MOM AND DAD, MOM MICHELLE AND DAD JEFF. THE BABY ACTUALLY CAN MOVE

HIS LEGS AND DOING WELL, WHICH IS, AGAIN, A GOOD SIGN.

THE LAST GRANT I'M GOING TO DISCUSS WITH YOU OR SHOW YOU THE INFORMATION ABOUT IS DR. WILLIAMS OF BOSTON CHILDREN'S HOSPITAL. HE'S CONDUCTING A SICKLE CELL DISEASE TRIAL IN ADULTS USING THE PATIENT'S OWN STEM CELLS GENETICALLY ENGINEERED TO REDUCE SICKLE HEMOGLOBIN STABLY AND PERMANENTLY AND SIMULTANEOUSLY USING FETAL HEMOGLOBIN RED CELLS OF THE AFFECTED INDIVIDUAL.

THIS TRIAL IS PART OF THE CIRM/NHLBI, WHICH IS THE NATIONAL HEART LUNG BLOOD INSTITUTE COLLABORATION. THE TRIAL IS RECRUITING PATIENTS IN CALIFORNIA AND OTHER U.S. SITES. MANUFACTURING OF THE CELLS IS ONGOING IN CALIFORNIA FOR THE CALIFORNIA PATIENTS.

SO IN CONCLUSION, THE TRANSLATION AND CLINICAL PORTFOLIO IS DIVERSE, COVERS MULTIPLE THERAPEUTIC AREAS. SEVERAL OF THE CLINICAL GRANTS ARE EARLY DEVELOPMENT CONSISTENT WITH CIRM DERISKING DEVELOPMENT. THE CLINICAL ADVISORY PANELS RESOLVED SEVERAL TECHNICAL, REGULATORY, AND STRATEGIC CHALLENGES, AND FACILITATED PARTNERSHIPS. EIGHT GRANTS PROGRESSED TO LATER STAGE DEVELOPMENT WORKING CLOSELY WITH US AND THE FDA WITH HALF REPEAT OF THE

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GRANTS OVER SEVERAL YEARS. HALF OF THE GRANTS REPEATED GRANTS OVER SEVERAL YEARS FOR THE SAME PROJECT.

I'M GOING TO LEAVE YOU WITH EVIE, THE CHILD WHO YOU SAW JUMPING IN THE AIR. HERE SHE IS -- YOU CAN SEE -- THE ADA-SCID THERAPY NINE YEARS OR SO AGO. THIS TIME YOU SEE HER JUMPING OR -- SORRY -- SURFING AND ENJOYING A NORMAL LIFE. SHE'S REALLY A ROLE MODEL FOR ALL THE OTHER TRIALS AND ALL THE OTHER PATIENTS. WITH THAT, I CONCLUDE AND THANK YOU.

CHAIRMAN TORRES: THANK YOU VERY MUCH, ABLA. I'LL TURN IT OVER TO SEAN. BUT JUST TWO THINGS. IT WAS SO INSPIRING I WAS BROUGHT TO TEARS WHEN THOSE CHILDREN RAN INTO OUR BOARDROOM AFTER WE HAD HEARD OF THEIR EXCITING DEVELOPMENT AND OVERCOMING THIS. THANK YOU TO DR. KOHN AND TO THE DOCTORS AT UCLA.

SECONDLY, IT'S PARTICULARLY INSPIRING FOR ME BECAUSE THE BARRERO FAMILY, ROSIE BARRERO, AND HER FAMILY WERE LONGTIME CONSTITUENTS OF MINE IN MY SENATE DISTRICT. AND I HADN'T SEEN THEM FOR YEARS UNTIL THEY APPEARED AT THE FIRST HEARING FOR THE FIRST GRANT TO DR. KLASSEN FOR THE PIGMENTOSA TRIAL. AND THERE SHE WAS BARELY ABLE TO SEE AND THEN

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RETURNING BACK, BEING TOTALLY ABLE TO RECOGNIZE ME FROM A DISTANCE. SO FOR ME THOSE MIRACLES HAVE JUST BEEN VERY INSPIRING. THANK YOU. SEAN.

DR. TURBEVILLE: THANK YOU, SENATOR. BEFORE I MOVE ON, I JUST WANT TO -- FIRST, ABLA, THANK YOU. THAT WAS WONDERFUL. AND HATS OFF TO YOU AND YOUR TEAM FOR PUTTING THAT MATERIAL TOGETHER.

I DO HAVE ONE QUESTION. AND THAT IS DO WE WANT TO PAUSE HERE FOR ANY QUESTIONS FOR ABLA BEFORE WE MOVE OVER TO THE ACCESS AND AFFORDABILITY?

DR. HIGGINS: I'D LIKE TO ASK A QUESTION, BUT I CAN'T GET MY HAND TO RAISE.

DR. CREASEY: GO AHEAD, DAVID. YOU DON'T HAVE TO RAISE.

DR. HIGGINS: OKAY. THANK YOU. I AGREE. ABLA, THAT WAS ONE OF THE MOST COHERENT PRESENTATIONS I'VE EVER HEARD OF OUR PORTFOLIO. I HAVE A QUESTION THAT REALLY IS FOR DR. MILLAN OR DR. CREASEY. AND THAT IS, AT THIS STAGE IN CIRM'S LIFE SPAN, ARE WE WHERE WE WANT TO BE OR WHERE WE SHOULD BE? ARE WE AHEAD? ARE WE BEHIND? GIVEN WHAT WE JUST HEARD FROM ABLA, HOW DO WE SCORE, IF YOU WILL, OUR PROGRESSION? THAT'S ONE. I HAVE A SECOND QUESTION TO FOLLOW UP.

DR. CREASEY: I CAN TRY TO GIVE YOU AN

ANSWER. I'M SURE DR. MILLAN WILL HAVE HERS TOO.

SO HAVING BEEN AT CIRM SINCE WE WERE CHARGED WITH RECRUITING 50 NEW CLINICAL TRIALS, AND WE DID 51, WE HAVE COME TO APPRECIATE THE IMPORTANCE OF BRINGING -- ADDING VALUE AND BRINGING, AGAIN, KEEPING PATIENTS FRONT AND CENTER AND HOW WE'RE GOING TO MAKE THOSE TRIALS AND THOSE TREATMENTS TO REACH ALL THE WAY TO APPROVAL.

PART OF THE FIVE-YEAR STRATEGIC PLAN WAS FOR US, AS I SHOWED YOU, IS TO GET SOME OF THESE PROGRAMS TO MOVE CLOSER IN DEVELOPMENT TO THE APPROVAL STAGE. AND SO WE ARE ON OUR WAY. WE ARE NOT THERE YET, BUT WE ARE HOPING WITH THE FIVE-YEAR TERM OF THE STRATEGIC PLAN THAT THINGS WILL PROGRESS TOWARDS THAT.

DR. HIGGINS: CAN I FOLLOW UP ON A QUICK QUESTION? ONE IS VERY SPECIFIC. IS THERE ANYTHING THAT THE BOARD CAN PROVIDE FOR YOU, DO FOR YOU, GET REMOVED FROM YOUR PATH OF OBSTRUCTION THAT WE SHOULD KNOW ABOUT FROM YOU?

DR. CREASEY: DAVID, YOU'RE VERY INTUITIVE AND SMART BECAUSE WE ARE THINKING ALONG THOSE LINES, BUT I'M GOING TO LEAVE THAT UP TO DR. MILLAN TO TALK ABOUT.

DR. HIGGINS: OKAY. MY LAST QUESTION,

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WHICH COULD BE FOR YOU, DR. CREASEY, OR DR. MILLAN. LET'S SEE. NO. NO. NO. I'M SORRY. THIS IS DIRECTED TOWARDS ART AND J.T. IF HE'S STILL THERE. AND THAT IS WHAT PARTING WORDS OF WISDOM, ART, J.T., WOULD YOU HAVE FOR THIS SPECIFIC GROUP AT CIRM NOW THAT YOU'RE GOING TO BE LOOKING AT IT FROM THE OUTSIDE IN?

CHAIRMAN TORRES: WELL, FIRST OF ALL, HAVING WRITTEN IT INTO THE INITIATIVE FOR 2020, I HAVE AND STILL HAVE TREMENDOUS EXPECTATIONS BECAUSE WE PUT TOGETHER AN INCREDIBLE GROUP. LOOK AT YOUR PEOPLE. YOU'RE INCREDIBLE. SO MANY WONDERFUL RESUMES, EXPERIENCES, HISTORY IN THE FIELD IN TERMS OF PATIENT ADVOCATES, IN TERMS OF SCIENTISTS, IN TERMS OF EDUCATORS, IN TERMS OF DOCTORS. I'M SO PROUD TO HAVE WORKED WITH YOU AND SO PROUD THAT YOU AGREED TO SERVE ON THIS WORKING GROUP BECAUSE IT IS THE INAUGURAL STEP AS WE MOVE FORWARD TO MAKE ACCESSIBILITY AND AFFORDABILITY FOR PATIENTS A PRIORITY.

THE OTHER IS WE INTEND TO WORK VERY CLOSELY WITH THE GOVERNOR'S OFFICE, WHO I'VE BEEN IN TOUCH MANY TIMES ALREADY, AS THEY BEGIN TO ESTABLISH THEIR OFFICE OF AFFORDABILITY, WHICH WAS CREATED BY THE LEGISLATURE AND SIGNED BY GAVIN IN LATE

SEPTEMBER, WHICH WILL CREATE AM OFFICE OF AFFORDABILITY WITHIN STATE GOVERNMENT. THAT LENDS MORE CREDENCE TO WHAT WE ARE DOING ON A CONSTITUTIONAL BASIS WITH OUR INITIATIVE PASSED BY THE VOTERS IN 2020.

SO MY EXPECTATIONS ARE VERY HIGH, AND I INTEND TO STAY IN TOUCH WITH AS MANY OF YOU AS I CAN EVEN ON AN INFORMAL BASIS. J.T.

CHAIRMAN THOMAS: THANK YOU, SENATOR.

SO I WOULD SAY ADVICE GOING FORWARD. SO I THINK WE ARE IN THE PROCESS OF WORKING ON DEVELOPING A STRATEGIC PLAN FOR THIS WORKING GROUP. AND THAT'S SORT OF A FIRST PRIORITY, TO GET THAT IN PLACE SO THAT ALL OF THE CONCEPTS THAT DERIVE FROM THAT CAN REFER BACK TO THAT AS WE GO ALONG HERE AND TO TRY TO DO THAT, AS THEY SAY IN THE LEGAL PROFESSION, ALL DELIBERATE SPEED, WHICH MEANS AS SOON AS WE CAN BECAUSE THESE ISSUES ARE VERY PRESSING. THERE ARE GOING TO BE THINGS THAT ARE NOT SIMPLE TO RESOLVE. THE ACCESSIBILITY ISSUES ON HOW WE'RE GOING TO REACH ALL THE CITIZENS OF CALIFORNIA PRESENT A DEFINITE LOGISTICAL CHALLENGE THAT'S GOING TO REQUIRE A LOT OF WORK AND ADVICE FROM THIS GROUP.

THE AFFORDABILITY ISSUES ARE EVEN TRICKIER BECAUSE THERE ARE LOTS OF VARIABLES TIED UP IN THAT

FOR WHICH WE DON'T REALLY HAVE ANY CONTROL, THAT ARE MARKET CONTROLLED, PAYOR CONTROLLED, AND THAT SORT OF THING. SO WE HAVE TO FIGURE OUT EXACTLY WHAT THE ROLE IS THAT WE CAN PLAY TO ADVANCE THE BALL AS WE PROCEED ON THE AFFORDABILITY FRONT.

I WANT TO THANK SENATOR TORRES FOR HIS WORK AS THE INAUGURAL, INIMITABLE CHAIR OF THIS WORKING GROUP AND GETTING THINGS GOING HERE. THANK YOU, ART, FOR ALL YOUR HARD WORK ON THAT. I'M GOING TO SAVE COMMENTS ON THE GRANDER SCALE FOR YOUR MANY YEARS ON CIRM FOR A LATER BOARD MEETING. BUT I WANT TO ALSO LET THE WORKING GROUP KNOW THAT OUR NEW VICE CHAIRMAN ELECT, MARIA BONNEVILLE, WHO IS ON THE CALL HERE, WHO WILL BE SWORN IN ON JANUARY 26TH, WILL BE THE NEW CHAIR OF THE WORKING GROUP, FILLING THE LARGE SHOES OF SENATOR TORRES. AND SO SHE WILL BE LEADING THESE MEETINGS FROM THIS POINT ONWARD.

SO THOSE ARE MY THOUGHTS. I DO WANT TO ECHO ART'S COMMENT THAT THE CALIBER OF ALL OF YOU WHO ARE ON THIS CALL AND A COUPLE OF THOSE, WE DON'T QUITE HAVE EVERYBODY HERE, BUT WHEN YOU TAKE A LOOK AT THIS GROUP, IT IS JUST EXCEPTIONALLY COMPETENT, BRINGING MANY DIFFERENT SKILL SETS TO THE TABLE. AND I'M SURE YOU WILL HAVE TREMENDOUS INPUT AS THE WORKING GROUP PROCEEDS ALONG FOR THE BENEFIT OF THE

PATIENTS OF CALIFORNIA. SO THANK YOU ALL VERY MUCH.

CHAIRMAN TORRES: SEAN.

DR. TURBEVILLE: I THINK TED GOLDSTEIN IS NEXT.

DR. GOLDSTEIN: HI THERE. CAN YOU HEAR ME?

DR. CREASEY: YES.

DR. GOLDSTEIN: I'M ON A WI-FI IN A BASEMENT OF A WHOLE FOODS, TRYING TO MAKE SURE WE MAINTAIN CONNECTIVITY.

SO THANK YOU VERY MUCH. I THOUGHT THE PRESENTATION WAS EXCITING AND INTERESTING AND A GOOD TASTE OF GREATER THINGS TO COME. BUT I WANT TO POINT OUT THAT THIS IS -- A TASTE IS NOT A MEAL. AND WE REALLY NEED, I THINK, AN IN-DEPTH REVIEW OF THE PORTFOLIO OF THE ENTIRE 47 OR 51 -- I'VE HEARD BOTH NUMBERS -- CLINICAL TRIALS THAT ARE ONGOING. AND I BELIEVE WE NEED TO UNDERSTAND THE MONEY AND THE IMPACT OF THESE DISEASES.

I THINK THAT THERE'S -- CLEARLY RARE DISEASES ARE VERY USEFUL FOR TEACHING US ABOUT MORE COMPLEX CONDITIONS AND WHERE WE CAN GET COOPERATION FROM THE FDA. BUT IF WE CURED 100 PERCENT OF ALL RARE DISEASES, WE WOULD IMPACT THE STATE NOT ENOUGH TO MEASURE. RIGHT. AND WE REALLY DO NEED TO

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UNDERSTAND THE IMPACT ON MORE COMMON DISEASES THAT AFFECT THE STATE.

ONE OTHER THING. YOU HAVE TWO OUTLIERS IN THE SIX YOU MENTIONED BY MY RECKONING, SPINA BIFIDA AND THE SICKLE CELL. I'M SORRY.

DR. CREASEY: GO AHEAD. SORRY.

DR. GOLDSTEIN: SO THOSE TWO CLEARLY HAVE GREATER IMPACT THAN THE RARE DISEASE COLLECTION. AND IT WOULD BE REALLY GREAT TO SORT OF UNDERSTAND THE POTENTIAL THERE. I BELIEVE THAT THE BIGGEST CRITICISMS PEOPLE HAVE MADE OF CIRM HAVE BEEN WHAT IS THE RETURN ON THE INVESTMENT SO FAR. RIGHT. AND THIS IS TRUE BOTH AT THE AGGREGATE LEVEL AND ESPECIALLY THE AFFORDABILITY LEVEL. AND WE NEED TO, I THINK, INCLUDE IN OUR ANALYSIS. YOU HAVE A BEGINNING OF A GOOD TEMPLATE TO LOOK AT THE CONDITIONS AND WHERE -- IT'S GREAT TO HAVE SOMETHING TO TALK TO, SO ALL POWER TO YOU, RIGHT. IT'S A GREAT FIRST STEP, BUT LET'S ELABORATE THE TEMPLATE WITH NUMBER OF PEOPLE IMPACTED, POTENTIAL NUMBER OF PEOPLE IN CONDITION, WHO HAVE THIS CONDITION, IN THE STATE AND IN THE NATION, IF THERE IS ASYMMETRY THERE. WHAT CAN WE -- WHAT IS THE EXPECTED IMPACT ON THE STATE BOTH IN TERMS OF LIVES AND DOLLARS?

I THINK AFFORDABILITY HAS A LOT TO DO WITH

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HOW WE ENABLE PEOPLE TO GAIN ACCESS TO THIS. AND SO THE FETAL CELL IMPACT IS A TREMENDOUS TECHNOLOGY AND WONDERFUL, BUT IT DOES NOTHING, OF COURSE, FOR EVERYBODY BORN. AND WE NEED TO UNDERSTAND WHERE IN THE PORTFOLIO EXISTING PEOPLE WHO SUFFER FROM SICKLE CELL ARE GOING TO BE MANAGED AND WHAT CAN BE DONE, IF ANYTHING. AND IT COULD EASILY BE THAT THE SCIENCE AND THE BIOLOGY MAKE IT THAT IT'S NOT FEASIBLE TO CREATE STEM CELL THERAPIES FOR PEOPLE ALREADY BORN.

THIS IS, I THINK, AS WE GROW THIS PROCESS ALONG, IT'S GOING TO BE VERY USEFUL FOR US TO ELABORATE THIS TEMPLATE AND STRUCTURE AND TO HAVE A BIRD'S EYE VIEW OF THE ENTIRE PORTFOLIO THAT SHOULD BE INCLUDED IN WHAT WE DO.

DR. CREASEY: THANK YOU FOR THAT. DR. GOLDSTEIN, THANK YOU. I JUST WANTED TO CLARIFY ONE THING. THE SPINA BIFIDA AND THE SICKLE CELL GRANTS BOTH HAVE DONE WELL, BUT THEY HAVE NOT SUBMITTED REQUESTS FOR AN ACCELERATED DESIGNATION TO THE FDA, BUT THEY ARE IN DISCUSSIONS WITH THE FDA. AND SO THE ONES THAT WE KNOW AND THEY'VE SHARED WITH US THAT THEY HAVE ACCELERATED APPROVAL WERE DESIGNATED ON THE SLIDES. SO JUST I WANT TO CLARIFY THAT.

RECALL ALSO ANY DESIGN OF A TRIAL, FOR

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EXAMPLE, WITH DR. WILLIAMS FOR SICKLE CELL, HIS DESIGN FOR HIS PHASE 2 TRIAL IS IN COLLABORATION WITH THE FDA AND MULTIPLE OTHER PARTIES. SO THAT'S ONGOING.

AS FAR AS -- I'M HAPPY TO SHARE THE REST -- WE HAVE FOUR DIFFERENT SICKLE CELL GRANTS IN CLINICAL. HAPPY TO SHARE THOSE ALSO. I PICKED HIM BECAUSE HE'S THE FARTHEST. AND SO THE OTHERS, HAPPY TO SHARE ALL THE DETAILS OF THE OTHER TRIALS AS WELL AS I UNDERSTAND IN TERMS OF, LIKE, THE ECONOMICS OF THE SITUATION, LIKE THE PREVALENCE OF THE DISEASE, HOW DO WE MANAGE ALL THAT, IT'S SOMETHING WE CAN ADDRESS AT A LATER TIME BECAUSE THAT WASN'T REALLY PART OF MY MANDATE HERE TODAY TO GIVE YOU A TASTE FOR WHAT'S GOING ON IN CLINICAL DEVELOPMENT. AND FOR US TO DISCUSS THE REST OF THAT, MOST LIKELY IN A CLOSED SESSION.

DR. GOLDSTEIN: I THINK IT WOULD BE VERY GOOD FOR US TO HAVE IN-DEPTH SUBCOMMITTEES THAT CAN FOCUS ON SOME OF THE MICRO DETAILS AND THEN BRING BACK THE LEARNINGS TO THE COMMITTEE AS A WHOLE.

DR. CREASEY: SOUNDS GOOD. THANK YOU. I'M EXCITED THAT YOU ARE THAT INTERESTED. THAT WILL BE AWESOME.

CHAIRMAN TORRES: I'M VERY SURE THAT MARIA

IS ALREADY THINKING ABOUT THAT AS WE'VE HAD THOSE DISCUSSIONS. GO AHEAD, SEAN. I THINK AMMAR IS MOOT. OH, THERE HE IS.

DR. QADAN: FIRST OF ALL, I WANTED TO THANK YOU, SENATOR, FOR YOUR SERVICE. AND I WANTED TO THANK JON AND WISH MARIA ALL THE BEST LEADING THIS GROUP.

THIS WAS A GREAT PRESENTATION, I THINK, IN MY DISCUSSIONS BEFORE WITH SEAN AND MARIA, I WANTED TO GET INTO THAT LEVEL OF DETAIL. I FEEL ANY ASSET THAT IS CURRENTLY IN PHASE 3, WHEN IT COMES TO HOW WE WANT TO DEMONSTRATE VALUE USING THE CLINICAL TRIAL PROGRAM, I NEED TO GET MORE DETAILS AROUND HOW WE ARE THINKING ABOUT THAT. AND WHEN I TALK ABOUT VALUE, IT'S NOT ONLY THE CLINICAL VALUE, BUT THE ECONOMIC VALUE. HOW WE ARE THINKING ABOUT THAT?

BECAUSE TO JON'S EARLIER COMMENT AROUND HOW WE WANT TO IMPACT PAYORS, IF WE ARE IN PHASE 3 AND WE'RE NOT CONSIDERING SOME OF THOSE ECONOMIC ANALYSES, WE ARE ALREADY LATE. AND SO IT WOULD BE GREAT IF, AT LEAST WHERE I CAN HELP, IS ANYTHING IN PHASE 2 OR PHASE 3 WE NEED TO HAVE A DEEPER DIVE AND MAKE SURE THAT WITHIN THE CURRENT CLINICAL TRIAL PROGRAM WE HAVE THE RIGHT ENDPOINTS ADDRESSED AND THE RIGHT PLAN IN PLACE TO ADDRESS THE VALUE FROM A

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PAYOR PERSPECTIVE.

AND, AGAIN, THANK YOU AND LOOKING FORWARD TO GETTING MORE DETAILS AROUND THOSE.

DR. CREASEY: MR. QADAN OR DR. QADAN, I WANT TO ASSURE YOU PHARMACOECONOMIC ANALYSIS HAS BEEN DONE ON SEVERAL OF OUR PROGRAMS BY EITHER THE GRANTEE OR WE ACTUALLY HAVE GIVEN A GRANT TO A CRO THAT HELPS IN GENERATING SOME OF THE INFORMATION, BUT ESPECIALLY TO THE PROGRAMS THAT HAVE ADVANCED FURTHER. SO WE ARE ON YOUR SIDE UNDERSTANDING THE PHARMACOECONOMICS OF THE GRANTS.

CHAIRMAN TORRES: MARIA AND ADRIENNE.

MS. SHAPIRO: I JUST WANTED TO SAY THAT SICKLE HAS TWO PHARMA COMPANIES WHO ARE CURRENTLY PREPARING TO GO TO FDA FOR APPROVAL, AND THAT WE, OUR GROUP, ARE PREPARING TO GO TO AN ISSA REVIEW. SO WE'LL HAVE A LOT OF DOCUMENTATION, ACTUAL OUR DOCUMENTATION, WHICH CHURNS THESE NUMBERS WHICH I THINK WILL BE VERY, VERY HELPFUL. SO I WILL KEEP EVERYBODY ABREAST OF THAT, AND THEN WE CAN TALK ABOUT LATER.

BUT THINKING ABOUT IT IN TERMS OF THE ECONOMIC, I'M GOING TO SAY, THE ECONOMIC IMPACT OF WHAT THIS RESEARCH IS DOING FOR ONE PARTICULAR GROUP, WITHOUT LOOKING AT WHAT THE PROMISE OF THAT

PARTICULAR -- THE SUCCESS OF THAT, LIKE ONE GENE KIND OF THERAPIES CAN DO IN OTHER PLACES, I THINK, IS SOMETHING THAT WE SHOULDN'T DO. WHENEVER WE TALK ABOUT IT, I THINK WE NEED TO ALSO REFERENCE THAT THIS IS A TECHNOLOGY AND WHAT OTHER THINGS IT'S GOING TO BE APPLICABLE TO. SO I DON'T WANT US TO LOSE SIGHT OF THAT.

AND THE OTHER THING IS IN OUR DISCUSSIONS WE HAVE TO TALK ABOUT REAL LIFE AND WHAT LESSONS LEARNED. AND ONE IS THAT TECHNOLOGY AND A PARADIGM SHIFT IN ANYTHING INITIALLY IS VERY EXPENSIVE. BUT AS WE IMPROVE UPON THE MODEL, THAT THAT EXPENSE THEN DECREASES. SO I REALLY WANT US TO HAVE THAT IN OUR CONVERSATIONS BECAUSE, AGAIN, WHAT WE ARE DOING NOW IN TERMS OF MEDICINE NOBODY HAS EVER DONE BEFORE. AND SO USING THE SAME MODELS TO ASSESS THE VALUE OF WHAT WE ARE DOING, I THINK, IS, I WONT' SAY INAPPROPRIATE, INAPPROPRIATE AT THIS POINT IN TIME.

SO AND I ALSO WANT TO SAY J.T. AND TO ART, THANK YOU, THANK YOU FOR ALL THAT YOU'VE DONE AND FOR HELPING ME FIND MY VOICE AND SUPPORT THAT YOU'VE GIVEN OUR COMMUNITY WHICH LITERALLY HAD NONE. WE HAD NO VOICE ON THE STATE LEVEL. WE HAD NO VOICE ON THE NATIONAL LEVEL. AND THE WORK OUT OF CIRM REALLY CREATED A PLATFORM FOR US. SO FOR A DISEASE FOR 120

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YEARS HAD NOTHING TO LIVE AT A TIME WHEN WE HAVE A CURE SO CLOSE, AND THE WORK THAT YOU GUYS HAVE DONE, AND CIRM HAS DONE, AND MARIA AND EVERYBODY ELSE, I JUST WANT TO SAY THANK YOU.

CHAIRMAN TORRES: THANK YOU, ADRIENNE. I THINK MARIA MILLAN HAD SOMETHING TO SAY, SEAN.

DR. MILLAN: SENATOR TORRES AND SEAN, HARLAN LEVINE HAS HIS HAND UP. SO I'M GOING TO GO AHEAD AND CEDE THE FLOOR TO HARLAN BECAUSE A LOT OF THE THINGS THAT I WANTED TO BRING UP ARE BEING BROUGHT UP ALONG THE WAY. THANK YOU SO MUCH.

CHAIRMAN TORRES: OKAY.

DR. LEVINE: THANKS, MARIA. SO A COUPLE OF THE POINTS I WAS GOING TO MAKE, I'M REALLY FRUSTRATED BECAUSE ADRIENNE MADE THE POINTS SO MUCH MORE ARTICULATELY THAN I COULD. I THINK IT'S REALLY -- WE NEED TO BUILD ON WHAT SHE JUST SAID TO US BECAUSE I UNDERSTAND THAT CIRM -- WE SIT HERE TODAY FEELING AS IF CIRM IS UNDER A LITTLE BIT OF PRESSURE TO DEMONSTRATE ITS ROI, BUT IT'S VERY EASY TO GET ON THE DEFENSIVE AND THEN LOSE SIGHT OF THE BIG PICTURE, WHICH IS WE ARE THINKING SHORT TERM IN TERMS OF -- BY THE WAY, I TOTALLY AGREE WE NEED TO BE THINKING ABOUT THE PAYOR PERSPECTIVE BECAUSE IN SOME CASES THERE'S AN OBJECTION, BUT WE SHOULDN'T

FALL INTO THE TRAP OF THINKING THAT THE PAYOR PERSPECTIVE IS THE ULTIMATE VOICE OF WHAT'S GOOD FOR SOCIETY.

I WOULD ARGUE THAT, HAVING BEEN ON THE PAYOR SIDE FOR MOST MY NONPRACTICING CAREER, I THINK WE SHOULD BE VERY CAUTIOUS OF LIKE DEFAULTING TO TAKING THEIR PERSPECTIVE IS YOUR ROLE. WE NEED TO REMEMBER THE POINT THINGS ARE VERY EXPENSIVE WHEN YOU START AND THEY COME DOWN IN PRICE. WE NEED TO THINK ABOUT THE SOCIETAL -- HUNDRED AND SOME ODD YEARS AGO PEOPLE WERE SAYING IT'S TOO EXPENSIVE TO PUT HOLES IN MOUNTAINS, BUT WE WOULDN'T HAVE A TRAIN SYSTEM, WE WOULDN'T HAVE AN ECONOMY WITHOUT THAT. AND I FEEL LIKE THIS IS A SIMILAR SITUATION TO THAT.

I ALSO THINK WE NEED TO TRACK HOW LEARNINGS FROM ONE DISEASE WILL LEAD TO OTHER DISEASE. WE CAN REMEMBER HOW EXPENSIVE MEDICORP WAS WHEN IT CAME OUT. WE CAN REMEMBER HOW DRAMATICALLY THE PRICE OF THE HEP-C DRUGS HAS COME DOWN IN A SHORT PERIOD OF TIME.

AND HERE'S THE POINT I WOULD MAKE IS WHEN PEOPLE LIKE ME MAKE THESE COMMENTS, WE IMMEDIATELY GET POLARIZED INTO SAYING, WELL, YOU DON'T CARE ABOUT AFFORDABILITY. OF COURSE, I DO. I'VE MADE MY WHOLE CAREER DRIVING AFFORDABILITY FOR THE

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POPULATION. BUT WE HAVE TO UNDERSTAND THAT THIS IS A TIME OF SCIENTIFIC EXPLOSION THAT'S JUST DIFFERENT. AND WHILE THE GOVERNMENT HAS ISSUES IT NEEDS TO DEAL WITH WHAT IT CAN AFFORD OR NOT, IF YOU LOOK AT THE ACA, IF YOU LOOK AT THE MARKET CAPS OF THE PAYORS, THE PBM'S, THE PHARMA COMPANIES, AND THEN THE MONEY THAT'S BEING TAKEN OUT BY THE MEDICAL GROUP FORMATION, THERE'S PLENTY OF MONEY IN HEALTHCARE TODAY TO AFFORD THESE THINGS. WE JUST NEED TO MAKE SURE THAT WE GET OUR VALUES CORRECT SOCIETALLY.

I'LL GET OFF THE BULLY PULPIT BECAUSE OF THE HOUR, BUT DON'T FALL IN THE TRAP OF BEING DEFENSIVE. WE ARE ADVANCING SCIENCE AND GOING TO IMPROVE HUMANITY OVER TIME, AND WE NEED TO GET THROUGH THE NEXT TEN YEARS, BUT ALL INDUSTRIES GO THROUGH THIS. AND THAT'S MY ADVICE. DON'T FALL INTO PAYOR TRAP WHEN YOU'RE THINKING ABOUT ONE QUARTER AFTER ANOTHER.

DR. TURBEVILLE: THANK YOU, HARLAN.

DR. MILLAN: I KNEW THAT HARLAN WOULD TOUCH SOME OF THE POINTS.

THIS IS JP MORGAN WEEK, AND WE ALSO HAD A LOT OF DIFFERENT WORKSHOPS AND KIND OF DEEP DIVES WITH MEMBERS OF THE COMMUNITY, INDUSTRY, ACADEMIA,

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DEVELOPERS. AND ONE OF THE THINGS THAT'S REALLY QUITE REMARKABLE IS THAT THE FIRST-IN-CLASS APPROVALS FOR CELL AND GENE THERAPY JUST HAPPENED UPON US LESS THAN FIVE YEARS AGO WITH THE FIRST CAR-T APPROVALS AND THE SPARK THERAPEUTICS FOR BLINDING EYE DISEASE AND THE GENE THERAPY FOR SPINAL MUSCULAR ATROPHY. WE'RE JUST STARTING TO SAY NOW IT'S A FIELD.

AND WHEN YOU TALK ABOUT VALUE OF CREATION AND ROI, THE TYPES OF INDICATIONS THAT CERTAINLY CIRM FUNDS, THE TYPES OF PROGRAMS WE FUND, THE VALUE PROPOSITION IS THAT THERE'S ACTUALLY NOTHING ELSE FOR THOSE DISEASE INDICATIONS. AND MANY IN THE FIELD, SCIENTISTS AND PHARMA AND DEVELOPERS ALIKE, ALL HAVE STATED VERY CLEARLY THAT THERE ARE SOME INDICATIONS, INCLUDING THE ONES WE ARE GOING AFTER, THAT ARE NOT ADDRESSABLE EXCEPT FOR GENE THERAPY OR EVEN CELL AND GENE THERAPY.

SO I THINK THAT THAT REALLY SETS IT APART FROM THE TYPE OF ANALYSIS ONE WOULD DO FOR A COVERAGE ANALYSIS THAT ONE WOULD DO FOR KIND OF A SMALL MOLECULE OR STANDARD BIOLOGICS. IT'S A NEW FIELD. SOME LIKEN IT TO THE MONOCLONAL ANTIBODY FIELD WHERE IT TOOK QUITE A WHILE FOR THAT TO REALLY EXPLODE, AND THEN THEY BECAME THE HIGHEST VALUE ROI

AND THE BIGGEST PRODUCTS OUT THERE IN THE MARKETPLACE.

BUT I WANTED TO ALSO SAY THAT THAT DOESN'T MEAN THAT WE DON'T DO ANYTHING ABOUT IT. I THINK THERE'S A TREMENDOUS OPPORTUNITY ACTUALLY, AND THAT'S WHY THE WISDOM OF SENATOR TORRES AND ADVISING THOSE WHO CRAFTED THE INITIATIVE AND THOSE WHO CRAFTED THE INITIATIVE IN MAKING SURE THAT CIRM, THROUGH YOUR GUIDANCE, WOULD BE ABLE TO FUND IMPORTANT RESEARCH IN HEALTHCARE ECONOMICS RESEARCH THAT COMBINES WITH REAL-WORLD EVIDENCE IN REAL TIME THROUGH OUR THERAPEUTICS DEVELOPMENT PORTFOLIO. AGAIN, YOU'RE RIGHT. IT'S JUST A TASTING MENU BECAUSE TO GO THROUGH THE OUTPUT OF THE 86 MOSTLY FIRST-IN-HUMAN CLINICAL TRIALS WOULD BE SOMETHING THAT WOULD REALLY TAKE A WHILE.

ANYWAY, I JUST WANTED -- THIS IS SOMETHING THAT WAS ANTICIPATED IN OUR STRATEGIC PLAN, AND THAT'S WHY THERE IS A SPECIFIC FIVE-YEAR GOAL TO CREATE A ROAD MAP, WHICH IS SOMETHING THAT CHAIRMAN THOMAS HAD ALLUDED TO EARLIER AND THAT SEAN TURBEVILLE HAD PRESENTED TO THIS GROUP AND WILL BE WORKING THROUGH. BUT PART OF THAT IS TO DO THE NEW TYPE OF RESEARCH THAT CIRM DOESN'T CURRENTLY FUND.

SO DR. HIGGINS, DAVID HIGGINS, OUR BOARD

MEMBER, HAD ASKED WHAT COULD THE BOARD HELP US WITH? AND WHEN WE ARE READY, IT'S TO LOOK AT DIFFERENT OTHER TYPES OF FUNDING OPPORTUNITIES THAT CAN BRING THIS FORWARD THAT'S NOT NECESSARILY OUR STANDARD PILLAR PROGRAMS AS WE HAVE IT. I TOOK A LOT OF TIME THERE, BUT SINCE SOMEBODY ASKED ME A QUESTION, I THOUGHT I'D TAKE THE OPPORTUNITY TO SHARE THOSE THOUGHTS. THANK YOU SO MUCH, SENATOR TORRES AND DR. TURBEVILLE.

DR. TURBEVILLE: THANK YOU, MARIA. I'M GOING TO HAND IT OVER TO ADRIENNE FOR THE LAST COMMENTS, AND THEN WE'LL PUNT OFF THE PRESENTATION ON ACCESS AND AFFORDABILITY.

MS. SHAPIRO: WAS MY HAND STILL UP? I DIDN'T MEAN FOR IT TO BE STILL UP. JUST ONE THING I HAVE TO SAY, AND CIRM IS PROBABLY TIRED OF HEARING ME SAY THAT, IS THAT I THINK, IN LOOKING AT NOT ONLY WHAT'S IN OUR PORTFOLIO, BUT LOOK OVERALL TO WHAT CIRM OR WHAT GENE THERAPY AND CELL TECHNOLOGY HAS BROUGHT TO THE STATE OF CALIFORNIA BECAUSE THERE'S A LOT OF OTHER THINGS THAT ARE STARTED HERE OTHER THAN JUST WHAT WE'VE SEEDED, BUT THEY'RE HERE BECAUSE OF WHAT WE'VE DONE. SO I ALSO WOULD LIKE TO HAVE THAT INCLUDED IN THIS DISCUSSION.

DR. TURBEVILLE: OKAY. THANK YOU. ALL

FASCINATING AND REALLY GOOD INSIGHT FROM THE ENTIRE TEAM. I THINK AT THIS POINT, ONE MORE, ABLA, I THINK, HAD HER HAND UP, AND THEN WE'LL START THE ACCESS AND AFFORDABILITY.

DR. CREASEY: I JUST WOULD LIKE TO THANK EVERYONE FOR THEIR COMMENTS. BUT I HAVE AS A SCIENTIST AND A CLINICAL PERSON WHO'S DONE A NUMBER OF TRIALS OVER THE YEARS, AND I WORKED ON MONOCLONAL ANTIBODIES AND MOVED THEM ALL THE WAY TO APPROVAL AND BEYOND, WHAT'S HAPPENING IN OUR FIELD IN CELL AND GENE THERAPY IS THE LOW HANGING FRUIT IS WHAT'S BEING WORKED ON IN ORDER TO DEMONSTRATE THE VALUE OF THE SCIENCE. ONCE THAT'S PROVEN TO BE SAFE AND EFFICACIOUS, THEN WE CAN APPLY IT TO THE MORE CHRONIC DISEASES.

THAT'S WHAT THE FIELD IS KIND OF SUFFERING FROM RIGHT NOW. WE ARE CAPTURED IN THAT KIND OF ARENA. BUT PLEASE BE ASSURED THAT WORKING IN AREAS OF CHRONIC DISEASE IS HIGH ON OUR PRIORITY, SUCH AS DIABETES, SUCH AS DIFFERENT CANCERS, SUCH AS NEUROLOGICAL DISEASES. SO THOSE ARE HIGH ON OUR LIST, AND WE'RE GOING AFTER THEM. IT'S A MATTER OF JUST SHOWING YOU WHAT WE HAVE ACCOMPLISHED THUS FAR WITH WHAT THE SCIENCE KNOWS HOW TO DO WELL AND ACCOMPLISH, MAINTAIN PATIENT SAFETY FIRST AND

FOREMOST AS THE KEY DRIVER, PLUS UNDERSTANDING REALLY THE PATHOGENESIS OF THOSE DISEASES AND HOW THEY EDUCATE US TO DO TRIALS FOR THE CHRONIC DISEASES THAT WE ARE ALL INTERESTED IN. THANK YOU.

MR. TORRES: SEAN, WE HAVE 30 MINUTES LEFT, SO GO TO IT.

DR. TURBEVILLE: YES, SIR. I'M GOING TO SHARE MY SCREEN. OKAY. IF SOMEBODY CAN GIVE ME A THUMBS UP THAT YOU CAN SEE THE SLIDES. THANK YOU, MARIVEL.

ALL RIGHT. WELL, FASCINATING DISCUSSION. THANK YOU, ABLA, FOR THE PRESENTATION. OBVIOUSLY YOU TEED UP A LOT OF QUESTIONS THAT LEAD INTO ACCESS AND AFFORDABILITY. AND J.T. BROUGHT UP SOME REALLY CHALLENGING COMMENTS AND QUESTIONS WITH RESPECT TO WHERE CIRM SITS AND HOW WE'RE GOING TO APPROACH METHODOLOGICALLY ACCESS AND AFFORDABILITY. QUESTIONS ABOUT PHARMACOECONOMICS, WHICH, OF COURSE, AMMAR AND I CAN TALK ABOUT, WHICH I THINK LEADS INTO THE DISCUSSION AND SOME OF THE SLIDES THAT I PUT TOGETHER TODAY.

SO, AGAIN, ABLA, I WANT TO THANK YOU FOR THAT BECAUSE THAT REALLY DID TEE OFF A BUNCH OF GOOD QUESTIONS. AND THOSE DEMONSTRATE ALL THE GREAT WORK THAT CIRM HAS DONE ON THE CLINICAL SIDE, ON THE

PORTFOLIO SIDE ACROSS THE CIRM ORGANIZATION.

SO IF YOU RECALL BACK IN, WELL, DECEMBER, WE OFFICIALLY KICKED OFF THE DEVELOPMENT OF OUR JOURNEY FOR OUR ROAD MAP OF ACCESS AND AFFORDABILITY. JUST TO REALIGN EVERYBODY AGAIN, AS STATED IN PROP 14 AND OUR FIVE-YEAR STRATEGIC PLAN, THIS ROAD MAP WILL INCLUDE A STRATEGY FOR GATHERING NOT ONLY THE NECESSARY DATA TO MAKE DECISIONS, BUT ALSO TO SUPPORT REIMBURSEMENT, ENGAGE WITH POLICYMAKERS AND REGULATORS, DEVELOP HEALTHCARE DELIVERY MODELS, WHICH IS A BIG ASK, BUT ALSO STRESS TEST SOME OF THOSE MODELS, NOT ONLY WITH OUR ALPHA CLINICS, BUT ALSO OUR FUTURE COMMUNITY CARE CENTERS OF EXCELLENCE.

SO IF YOU RECALL, I SHARED EVERYBODY THIS SLIDE. THIS IS THE SLIDE THAT FOR THE MOST PART HAS BEEN AAWG BLESSED. WE ARE TARGETING FOUR, FOR THE MOST PART, STRATEGIES, AND THIS IS SOMEWHAT OPEN FOR DISCUSSION. WE ARE CASTING A WIDE NET AS YOU CAN SEE FOR RIGHT NOW. THE FOUR STRATEGIES, AGAIN, THAT WE WANT TO FOCUS ON OUT OF THE GATE ARE THE REIMBURSEMENT THAT HAS BEEN TOUCHED ON JUST RECENTLY; LIMIT PATIENT EXPENSES, PARTICULARLY OUT-OF-POCKET EXPENSES, WHICH I'LL TALK ABOUT IN A FEW MINUTES; LOTS OF NEW INTEL IN THE LAST THREE

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WEEKS ON PAYOR MODELS, THOSE NEW PAYOR MODELS; CERTAINLY WANT TO BE INTO, OF COURSE, WITH THE STATE POLICY ISSUES THAT HAVE, AGAIN, JUST BEEN DISCUSSED EARLIER. AND, FINALLY, AGAIN, EXPAND ON THE CLINICAL INFRASTRUCTURE AND HOW WE MIGHT BE ABLE TO PLAY IN THAT SPACE, AGAIN PIGGYBACKING OFF ALL THE GREAT WORK THAT'S BEEN DONE WITH THE ALPHA CLINICS AND THEN, OF COURSE, THE VISION THAT WE HAVE FOR THE COMMUNITY CARE CENTERS OF EXCELLENCE.

SO I'M SHOWING THIS BECAUSE IT IS A LARGE, IF YOU WILL, MAP. WE ARE SETTING SORT OF A WIDE STRATEGY HERE, BUT IT IS ALSO OPEN FOR DISCUSSION FOR STRESS TESTING. I WANT TO START LARGE AND START KIND OF FIDDLING DOWN TO THE BASICS THAT MIGHT HAVE THE BIGGEST IMPACT FACTOR, IF YOU WILL, FOR EACH ONE OF THESE STRATEGIES.

SO THE PURPOSE OF TODAY'S PRESENTATION IS REALLY JUST TO PROVIDE SOME INPUT THAT WE'VE GATHERED IN THE LAST COUPLE WEEKS. THERE'S A COUPLE OF ASKS THAT I HAVE OF THE AAWG, WHETHER WE'RE ON TARGET, WHETHER THERE'S SOMETHING WE ARE MISSING IN THE LAST COUPLE WEEKS WE HAVEN'T THOUGHT ABOUT FROM THE STRATEGY STANDPOINT. NOT SO MUCH TACTICALLY. WE CAN GET INTO THAT LATER.

AND THEN I ALSO WANT TO TALK A LITTLE BIT

ABOUT SOME INTERESTING DATA FROM THE CAR-T SPACE. THAT MIGHT BE A BENCHMARK FOR US. IT'S NOT GENERALIZABLE TO EVERYTHING THAT WE'RE DOING AT CIRM; BUT CERTAINLY, AS YOU KNOW, CAR-T, AND MANY PEOPLE ON THIS PANEL HAVE A LOT OF EXPERIENCE WITH THIS, WE'RE SIX TO SEVEN YEARS DEEP NOW INTO THE CAR-T SPACE, NOT ONLY ON THE CLINICAL SIDE, BUT ALSO ON THE OPERATIONAL SIDE. SO LOTS OF QUESTIONS AND FEEDBACK THAT WE'RE GOING TO ASK OF THE AAWG: WHETHER WE ARE TARGETING THE RIGHT STRATEGIES AND THAT THE TACTICS THAT WE ARE USING GO AFTER THOSE.

SO I DO WANT TO SET THE STAGE BECAUSE IT'S FASCINATING. J.T. MENTIONED THIS. THE LANDSCAPE IS MOVING SO QUICKLY. I THINK AMMAR TOUCHED ON THIS AS WELL. JUST AS AN EXAMPLE, LAST YEAR THERE WERE APPROXIMATELY 500 UNIQUE CELL AND GENE PRODUCTS IN EITHER PHASE 1, PHASE 2, OR PHASE 3 TRIALS. AND THEY'RE ALL, FOR THE MOST PART, PERSONALIZED. SO THEY ALL HAVE THEIR OWN OPERATIONAL CHARACTERISTICS THAT ARE REALLY IMPORTANT FOR US TO UNDERSTAND.

ON SATURDAY WE HAD A VERY LARGE REGULATORY MEETING HOSTED, OF COURSE, AT UCSF, WHICH WAS REALLY INSIGHTFUL. GOT TO SPEAK TO QUITE A FEW FDA FORMER CHIEFS AND PICK THEIR BRAINS. THE FDA WILL DECIDE ON A MINIMUM OF 13 CJT'S THIS YEAR. THREE WILL BE

DECIDED IN EUROPE. AND IT'S EXPECTED OR ANTICIPATED THAT 50 TO 75 CELL AND GENE THERAPIES ARE EXPECTED TO BE APPROVED IN THE U.S. BY 2030. SO THAT IS AN ENORMOUS OPERATIONAL LIFT, NOT ONLY FROM THE CLINICAL SIDE, THE FDA'S OF THE WORLD, THE COMPETENT AUTHORITIES, BUT REALLY THINKING THROUGH THE OPERATIONS. THAT'S WHERE I THINK THERE MAY BE SOME RATE LIMITING STEPS.

AS I'M SORT OF A NEWBY, TO BE HONEST WITH YOU, 11 MONTHS DEEP, IF YOU GO BACK AND LOOK AT THE FIVE-YEAR STRATEGIC PLAN AND EVERYTHING THAT WE'VE THOUGHT THROUGH, AND I CAN THROW SOME NAMES OUT WITH RESPECT TO SHYAM AND EVERYBODY THAT'S THINKING THROUGH THE MANUFACTURING PROCESS. WHEN YOU START LINING THESE UP, IT REALLY DOES MAKE SENSE IN TERMS OF WHERE WE THINK THE MODEL IS GOING TO BE THREE TO FIVE YEARS FROM NOW. SO THERE'S BEEN A LOT OF HEADWAY, BUT THERE ARE A NUMBER OF CHALLENGES AND BARRIERS CERTAINLY FOR THE STATE THAT ARE BEING ADDRESSED NOW, AND WE STILL HAVE SOME WAYS TO MAKE IMPROVEMENTS.

THERE IS SOMETHING I WANT TO BRING TO EVERYBODY'S ATTENTION. THIS IS AN INTERESTING SURVEY THAT WAS RECENTLY DONE, JUST LITERALLY MAYBE A COUPLE OF MONTHS AGO. AND THE SURVEY WAS DONE ON

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A NUMBER OF CAR-T SITES, APPROXIMATELY 22 OUT THERE. AND THEY ASKED A NUMBER OF QUESTIONS WITH RESPECT TO REIMBURSEMENT AND WHETHER OR NOT REIMBURSEMENT RESULTS IN FEWER PATIENTS HAVING ACCESS TO THESE THERAPIES.

SO THERE ARE A COUPLE OF MYTHS. AND EVEN I, IT'S TOUGH TO KEEP UP WITH ALL THE INFORMATION. BUT THE FIRST MYTH IS THAT PROVIDERS OF THESE THERAPIES ARE LOSING MONEY, THAT IT WAS A SIGNIFICANT BARRIER TO PATIENT ACCESS. AND I THINK, DEPENDING ON WHAT COHORT YOU TEST, IN THE BEGINNING THAT MAY BE TRUE. BUT WHAT WE ARE FINDING FROM THE SURVEY IS THAT 90 PERCENT OF THE SURVEYED CENTERS REPORT EARNING A PROFIT OR AT LEAST BREAKING EVEN ON CAR-T ADMINISTRATION. AND ADDITIONALLY, 75 PERCENT OF THESE CENTERS ARE PROFITING OR BREAKING EVEN ON INPATIENT MEDICARE PATIENTS. SO POINT BEING HERE IS ACTUALLY THIS MYTH HAS BEEN SORT OF DEMOLISHED TO SOME EXTENT IN THAT THERE'S BEEN GREAT PROGRESS. AND TO BE FAIR, ECONOMICS DOES DRIVE ACCESS AND AFFORDABILITY. IT IS A SIGNIFICANT COVARIATE IN THAT REALM. SO THAT WAS MYTH NO. 1.

WHAT THEY ATTRIBUTE THIS TO IS THAT, ONE, IMPROVED HOSPITAL BILLING. WE JUST HEARD YESTERDAY ABOUT MEDICAID NOW PUTTING IN NEW INFRASTRUCTURE ON

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THE REIMBURSABLE SIDE THAT ALLOW INFUSIONS AT A PHYSICIAN SITE. SO THAT'S GREAT PROGRESS FOR PATIENTS.

THE OTHER THING ABOUT THIS SURVEY THAT'S INTERESTING IS, IN ORDER TO BE SUCCESSFUL, MANY SITES MENTION THAT THEY HAD TO HAVE A COMBINATION OF NOT ONLY PRIVATE BUT ALSO PAYOR REIMBURSEMENT. THAT IT WAS NEITHER ONE OR THE OTHER, THAT THE COMBINATION REALLY WAS SORT OF THE SECRET SAUCE, IF YOU WILL, FOR ACTUALLY BEING SUCCESSFUL, TO BE ABLE TO HAVE THERAPY FOR PATIENTS THAT ARE CERTAINLY IN ONE CATEGORY AND, OF COURSE, HAVING ACCESS TO THE PATIENTS OR ACCESS TO THERAPIES TO PATIENTS ON THE OTHER SIDE OF THE SPECTRUM. SO THERE'S THAT BALANCE.

AND FURTHERMORE, AS HARLAN PROBABLY WOULD CONCUR GIVEN THAT HE'S A SUBJECT MATTER EXPERT IN THIS AREA, APPROVED DEFICIENCY. SO FEWER CLINICAL COMPLICATIONS NOW. WE'RE SEEING SHORTER INPATIENT STAYS. BETTER FAMILIARITY WITH THE SAFETY PROFILE AND PRODUCT. AND FEWER HOSPITAL STAYS FOR PATIENTS.

SO THIS IS INTERESTING INTEL FOR US THAT I WANT THE AAWG TO CONSIDER AS WE START CONSIDERING OUR MODEL FOR ACCESS AND AFFORDABILITY.

MYTH NO. 2 IS INTERESTING. SO THAT WAS,

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HEY, LOOK, IS OUTPATIENT ADMINISTRATION FEASIBLE FROM AN ECONOMIC STANDPOINT AND OPERATIONAL STANDPOINT? AND WHAT THEY FOUND IS THAT ON AVERAGE 15 TO 20 PERCENT OF CASES ARE BEING ADMINISTERED ON AN OUTPATIENT SERVICE. AND START THINKING ABOUT THROUGH THIS WITH RESPECT TO THE COMMUNITY CARE CENTERS OF EXCELLENCE. THE FACT THAT THERE'S PRECEDENCE NOW, THERE'S A PUSH OUT TO THE OUTPATIENT SETTING IS SOMETHING THAT WE SHOULD CONSIDER FOR THE COMMUNITY CARE CENTERS OF EXCELLENCE AND MAKING SURE THAT THOSE ORGANIZATIONS, THOSE INSTITUTIONS HAVE THE OPERATIONAL SUPPORT TO BE ABLE TO SORT OF PLUG AND PLAY, IF YOU WILL, THE OUTPATIENT CAR-T INFUSIONS. AND ALSO PREPPING THEM FOR MUCH MORE ADVANCED GENE THERAPY IN THE FUTURE.

SO, AGAIN, THIS OBSERVATION DOES ALIGN WITH THE CC INITIATIVE, AGAIN, NOT ONLY PROVIDING RESOURCES FOR THE RESEARCH SIDE, BUT, AGAIN, ALSO FOR THE COMMERCIAL APPROVALS THAT WE ANTICIPATE, I MENTIONED EARLIER, THAT WILL BE NEEDED IN THE RURAL COMMUNITIES.

AND MYTH NO. 3 WAS MORE RELATED TO INDUSTRY. AND JUST TO BACK UP A LITTLE BIT, THIS SURVEY WAS DESIGNED BY AN ORGANIZATION THAT IS WELL-KNOWN IN THE INDUSTRY. IT'S CALLED ZS. THEY

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ARE SORT OF A MARKET ACCESS THINK TANK. I DO ENCOURAGE THEM, HOPEFULLY, TO PUT THIS INFORMATION IN THE PUBLIC DOMAIN, BUT IT IS BY FAR THE MOST ROBUST SURVEY INFORMATION THAT WE HAVE TO DATE ON THE CAR-T CENTERS IN THE UNITED STATES.

BUT THE MYTH 3 WAS REALLY, HEY, IS INDUSTRY HELPING WITH ACCESS AND AFFORDABILITY? IS THERE PUSHBACK? AND WHAT THEY FOUND IN GENERAL, THAT, AND NOT SURPRISING, MOST OF THE SITES ARE RECEPTIVE, NOT ONLY OF INDUSTRY ASSISTANCE, BUT ALSO THE PUBLIC SIDE ASSISTANCE AS WELL.

ONE THING THAT KEEPS COMING UP, AND THIS IS BY NO MEANS ANOTHER PLUG FOR THE PATIENT SUPPORT PROGRAM, BUT THE SITES DID VALUE THE MANUFACTURERS AND, MORE IMPORTANTLY, THE PATIENT SUPPORT PROGRAM, HOW ROBUST THOSE PROGRAMS ARE IN HELPING THESE SITES GET THE PATIENTS THROUGH THAT PATIENT JOURNEY.

SO IT'S SOME INTERESTING INTEL. WE CAN GET INTO SOME GREAT DETAIL HERE, BUT I DO ENCOURAGE YOU TO TAKE A LOOK AT THE SURVEY. IT COULD BE A GOOD MODEL FOR US MOVING FORWARD. I'LL LEAVE THAT OPEN FOR THE END DISCUSSION OF WHETHER OR NOT THIS IS SOMETHING THAT WE SHOULD CONTINUE TO FOCUS ON, PUTTING MORE EFFORTS, RESEARCH EFFORTS, INTO THIS EXPANSION, AND HOW THAT MAY PLAY OUT WITH RESPECT TO

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ACCESS AND AFFORDABILITY.

ANOTHER THING I WANT TO STRESS TEST WITH THE AAWG IS MAKING SURE THAT WE ARE FOLLOWING THE PATIENT JOURNEY. AND THIS IS SOMEWHAT NEW FOR CIRM TO BE HONEST WITH YOU. AND AMMAR MENTIONED THIS. THIS DOES PLAY INTO THE PAYOR PATHWAYS AND KIND OF REVERSE ENGINEERING THE PAYOR PATHWAY, THE PATIENT JOURNEY, AND WHAT THAT'S GOING TO LOOK LIKE ALL THE WAY FROM THE CLINICAL SIDE.

SO I'M HOPING THAT -- AND THIS IS JUST AN EXAMPLE OF WHAT A PATIENT JOURNEY WOULD LOOK LIKE ALL THE WAY FROM WHEN THEY SEE THE SPECIALIST, TO THE DIAGNOSTIC TEST, THE OUT-OF-PATIENT POCKET EXPENSES THAT TAKE PLACE FOR EACH PART OF THAT JOURNEY. BUT I WANT TO MAKE SURE THAT AT LEAST THIS EXERCISE IS IMPORTANT TO THE AAWG. IT DOES PROVIDE US GOOD INSIGHT IN TERMS OF WHERE THE POTHOLES ARE, WHERE THOSE SORT OF MILESTONES THAT WE MIGHT BE ABLE TO IMPACT FOR PATIENTS, NOT ONLY ON THE PAYOR SIDE, BUT ALSO ON THE OBSERVATIONAL SIDE. SO I DO WANT TO STRESS TEST THAT WITH THE AAWG GIVING GUIDANCE AT THE END THE DISCUSSION, WHETHER WE'RE SPOT ON OR MAYBE WE SHOULD PULL BACK AWAY FROM THE PATIENT JOURNEY.

THERE ARE A COUPLE THINGS THAT MANY OF YOU

ARE FAMILIAR WITH THAT WE'VE HEARD FROM THE COMMUNITY. ONE IS THAT CLINICIANS WOULD LIKE TO SEE NOT ONLY THE WORK-UP OF THE PATIENT AT THE SAME SITE AT WHICH THE INFUSION TAKES PLACE.

ANOTHER AREA IS THE COMPLEX PRIOR AUTHORIZATION. THIS IS VERY COMPLICATED AS MANY OF US KNOW. IT TAKES A SUBJECT MATTER EXPERT TO TAKE A PATIENT AND MAKE SURE THAT THEY GET THE PRIOR AUTHORIZATION FOR THE CELL AND GENE THERAPY. AND THAT IS ANOTHER POTENTIAL, AND I USE THE TERM "DELIVERABLE," BUT CERTAINLY SOMETHING THAT WOULD FALL UNDER THE PATIENT SUPPORT PROGRAM.

SO WE'LL COME BACK TO THIS AT THE END OF THE DISCUSSION. I'M COGNIZANT OF TIME. I WANT TO RUN THROUGH THIS AS QUICKLY AS POSSIBLE.

NOW, WHAT'S INTERESTING ON THE PAYOR SIDE, WHAT WE'VE HEARD FROM PAYORS, AND THIS GOES TO A COUPLE COMMENTS JUST EARLIER, MOST PAYORS CURRENTLY USE TRADITIONAL TECHNIQUES SUCH AS FORMULARY OR UTILIZATION MANAGEMENT TOOLS TO MANAGE CELL AND GENE THERAPIES. WHAT MANY PAYORS ARE LOOKING AT ARE FORMER MODELS. HEPATITIS C IS A GREAT EXAMPLE. SOMEONE MENTIONED THAT EARLIER. THEY ARE USING THAT MODEL AND TRYING TO MODEL THAT WITH THE CELL AND GENE THERAPY SPACE, AND THAT DOESN'T NECESSARILY

FIT. SO THERE ARE NEW MODELS THAT ARE BEING DEVELOPED AS I'M GIVING THIS PRESENTATION THAT I THINK THE AAWG NEEDS TO BE AWARE OF IN TERMS OF -- AND IT GOES TO THE PATIENT JOURNEY, BUT ALSO THE PAYOR JOURNEY THAT TAKES PLACE BECAUSE WE DO HAVE FAIRLY LARGE INVESTMENTS IN THESE ASSETS. AND UNDERSTANDING THAT WHOLE LIFE CYCLE MANAGEMENT IS CRITICAL.

NOW, INTERESTING ENOUGH, WHAT WE ALSO HEARD HAD FROM PAYORS IS THEY PLAN TO LEVERAGE REINSURANCE AS WELL AS INCREASE THEIR USE OF ALTERNATIVE PAYMENT MECHANISMS OR VALUE-BASED OUTCOMES. I'VE TALKED ABOUT THIS A COUPLE OF TIMES, BUT THIS IS NEW TO US. WHAT WE ARE HEARING FROM THE PAYORS IS THAT THEY'RE TRYING TO FIND INSURANCE ON TOP OF THE INSURANCE SPECIFICALLY FOR CELL AND GENE THERAPIES. AND THE REASON FOR THAT IS THAT UPFRONT HEAVY BURDEN FOR THE PAYORS WITH THOSE FRONT COSTS, THOSE FRONT INFUSIONS, WHICH YOU'VE HEARD THE NUMBERS BEFORE, WHICH ARE PHARMACOECONOMICALLY SUPPORTED, BUT NONETHELESS ARE A BIG IMPACT FACTOR FOR INSURANCE COMPANIES.

SO I WON'T GET INTO GREAT DETAIL. I TALKED ABOUT THE ANNUITY BASED, OUTCOMES BASED, OUTCOMES BASED REBATES. ALL OF THESE ARE CURRENTLY

BEING USED AS WE SPEAK WITH THE CELL AND GENE THERAPY SPACE.

NOW, THERE'S ANOTHER AREA THAT WE MIGHT BE ABLE TO FOCUS ON. AND THIS IS SOMEWHAT THEORETICAL, BUT THE GOVERNMENT IS STEPPING UP TO THE PLATE. THEY ARE TAKING A BIGGER INITIATIVE IN NOT ONLY HELPING ON THE FEDERAL SIDE WITH RESPECT TO REIMBURSEMENT, BUT ALSO AT THE STATE LEVEL. AND THIS IS WHERE I THINK SENATOR TORRES AND PEOPLE AT THE AAWG WHERE WE COULD WORK WITH THE STATE AND STARTING TO THINK THROUGH HOW THE FEDERAL PROGRAMS MIGHT BE ABLE TO HELP THE STATE, HOW THE STATE CAN HELP WITH POTENTIAL DEFICIENCIES ON THE, AND MANY OF YOU, AGAIN, ARE SUBJECT MATTER EXPERTS, THAT HIGH BURN RATE THAT'S GOING TO TAKE PLACE.

GOLDSTEIN MENTIONED IT EARLIER. THE BUDGET IMPACT OF CUMULATIVE, ALL OF THE ORPHAN DISEASES REALLY STILL IS A DROP IN THE BUCKET WHEN WE THINK ABOUT THE CAR-T'S THAT ARE COMING, THINK ABOUT SOLID TUMOR, PARTICULARLY ONCOLOGY SPACE, WHICH, OF COURSE, CIRM HAS INVESTED IN AS WELL. SO LOTS OF THINGS TO THINK ABOUT AND LOTS OF OPPORTUNITIES FOR US TO BRING TO THE AAWG.

AND THEN FINALLY, I DO WANT TO GET INPUT ON WHEN WE THINK ABOUT ACCESS AND AFFORDABILITY.

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AND THERE'S BEEN LOTS OF DISCUSSION JUST RECENTLY AT JP MORGAN, A NUMBER OF SCIENTIFIC CONGRESSES, AND THAT IS GETTING OUT TO THE COMMUNITY, THAT OUTPATIENT. WHAT DOES THAT LOOK LIKE AND WHAT IS THAT GOING TO TAKE? THIS GOES BACK TO AMMAR'S COMMENT ABOUT PHARMACOECONOMICS, BUT ALSO ACCESS, SPEED TO TREATMENT. AND SO THIS IS SOMETHING THAT WE WANT TO CONTINUE TO INVESTIGATE, BRING BACK TO THE AAWG, AND ALSO INCORPORATE -- AS YOU MAY KNOW, WE HAVE AN ADDITIONAL WORKING SESSION NEXT WEEK WITH COMMUNITY CARE CENTERS OF EXCELLENCE -- GATHERING THAT CLINICAL AS WELL AS THE COMMUNITY INFORMATION THAT WE CAN APPLY TO THAT CONCEPT PLAN THAT GEOFF IS DRIVING. SO ABSOLUTELY CRITICAL. WOULD LOVE TO GET YOUR PEOPLE'S INPUT ON THE OUTPATIENT SETTING WITH RESPECT TO CAR-T AND MORE IMPORTANTLY EVEN THE GENE THERAPY SPACE.

CHAIRMAN TORRES: BE SURE TO CALL ON JAMES BECAUSE HE BRINGS SUCH EXPERTISE FROM COVERED CALIFORNIA IN THIS WHOLE ARENA. SO HE'S GOING TO BE INVALUABLE TO YOU.

DR. TURBEVILLE: ABSOLUTELY. I APOLOGIZE.

SO I WANT TO REALLY KEEP THIS SOMEWHAT SHORT. IN GENERAL, I WANT TO MAKE SURE THAT WE ARE APPROACHING THIS THE RIGHT WAY. I'VE SHOWN YOU THAT

MAP. AGAIN, OUR VISION IS THAT WE'RE GOING TO TRY TO DO AS MUCH DUE DILIGENCE AS WE CAN AND BRING THIS BACK TO THE AAWG FOR COMMENTS, QUESTIONS, WHERE WE THINK THE SWEET SPOT IS, WHERE WE THINK THE OBSTACLES ARE TOO DIFFICULT FOR A SMALL ORGANIZATION LIKE US, BUT REALLY LOOK TO THAT FEEDBACK.

AND A SECOND QUESTION OF THE AAWG. ARE WE MISSING ANY STRATEGIES? I MENTIONED THOSE FOUR TO FIVE STRATEGIES WE ARE TARGETING. I DON'T WANT TO BOIL THE OCEAN. WE HAVE TO BE VERY CLEAR ON WHAT WE ARE GOING GO AFTER FOR THE NEXT FIVE TO SIX MONTHS AND HOW THIS CAN BE A SUCCESSFUL PROGRAM FOR ACCESS AND AFFORDABILITY FOR ALL PATIENTS, PARTICULARLY IN CALIFORNIA.

SO WITH THAT, LET ME PAUSE AND FIRST JUST SEE IF THERE'S ANY RESPONSE TO THE SLIDES THAT I PRESENTED AND, MORE IMPORTANTLY, THE TWO QUESTIONS. ARE WE APPROACHING THIS IN THE RIGHT WAY? ARE THERE OTHER METHODOLOGIES THAT WE SHOULD CONSIDER? AND THEN, FINALLY, ARE THERE ANY STRATEGIES THAT YOU THINK WE MAY BE MISSING? SO I'LL OPEN THAT UP FOR DISCUSSION, AND I WANT TO THANK YOU FOR THE OPPORTUNITY TO PRESENT.

MS. BONNEVILLE: HARLAN AND SOME HANDS RAISED OF YOU WANTED TO CALL ON ANYONE.

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DR. LEVINE: I THINK AMMAR WAS FIRST. I'LL JUST JUMP IN.

MS. BONNEVILLE: THANKS, HARLAN.

DR. QADAN: THANKS, SEAN. I AGREE WE ARE -- I AGREE. THANK YOU, SEAN. WE ARE INTRODUCING DIRECT STRATEGIES. MY SUGGESTION IS PROBABLY, IN ORDER TO GET THE VALUE OUT OF THE PARTICIPANTS WITHIN THIS GROUP, IS PERHAPS WE NEED TO SEPARATE THE WORK ON THE PATIENT AFFORDABILITY AND DRIVING PATIENTS TO CLINICAL TRIALS FROM DEMONSTRATING VALUE FROM A PAYOR PERSPECTIVE BECAUSE THOSE TAKE TWO DIFFERENT EXPERTISE TO MANAGE THOSE. AND THAT MIGHT NOT BE THE BEST USE OF PEOPLE'S TIME TO TACKLE BOTH ISSUES. SO THAT SPECIALIZATION MIGHT BE NEEDED.

THE SECOND POINT I WANTED ALSO TO SAY AT JP MORGAN ALSO THERE WERE SESSIONS BY BIOMER AND BY VERTEX. AND THEY ADDRESSED SOME OF THE EMERGING INNOVATIVE CONTRACTS AND SOME OF THE EMERGING ALSO REQUIREMENTS BY PAYORS TOWARDS THE DURABILITY OF RESPONSE AS A PREQUALIFIER. AND SOME OF THE WORK THAT ALSO HAVE BEEN SHARED AROUND MANY OF THE SMALL PAYORS COMING TOGETHER AND CREATING LIKE A MULTIPLE ORGANIZATION TYPE OF CONTRACT WITH ONE MANUFACTURER, WHICH I FELT THAT THIS WAS REALLY A GREAT IDEA TO

THINK ABOUT SO THAT THIS WOULD ENABLE ALSO ADOPTION BY THOSE PAYORS WHO WILL BE RELUCTANT TO ADOPT INITIALLY.

FINALLY, I WANT TO MAKE JUST A FINAL COMMENT. DEFINITELY I AGREE WITH HARLAN AROUND WE DO NOT WANT ALSO TO BE CHASING A GHOST. AND SOME OF THE REQUIREMENTS OF PAYORS, AND MARIA ALSO ALLUDED TO THAT, MIGHT NOT BE REALISTIC. BUT SOME OF THOSE ALSO WE NEED TO ADDRESS COMMONALITIES BETWEEN WHAT'S HAPPENING IN THE U.S. AND OUTSIDE THE U.S. WHILE I LOOK AT THE U.S., ESPECIALLY COMMERCIAL PAYORS, WITH SKEPTICISM BECAUSE THOSE ARE FOR-PROFIT ORGANIZATIONS. I SEE ALSO SOME OF THE SIMILAR TRENDS THAT WE SEE IN THE U.S. OUTSIDE THE U.S. WHERE THE PAYOR IS THE GOVERNMENT. AND SO I THINK THOSE NEED TO BE ADDRESSED. THANK YOU.

DR. TURBEVILLE: THANK YOU. I THINK PAT LEVITT WAS NEXT. PAT.

DR. LEVITT: SO, SEAN, YOU PROBABLY KNOW WHAT I'M GOING TO SAY. THAT SURVEY, WHICH IS AN INTERESTING SURVEY ABOUT MITT BUSTERS WAS, I'M CERTAIN FROM READING THE ARTICLE, IT WAS A SURVEY OF ALL ADULT MEDICAL CENTERS. AND SO THIS IS NOT MY OPINION NOW. THERE'S BEEN ANOTHER PIECE, I SENT IT TO SEAN, WRITTEN IN THE *NEW YORK TIMES*, AND THE

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TITLE OF THE ARTICLE WAS "WHY SAVING CHILDREN IS BAD FOR BUSINESS." AND SO THIS IS A PERSISTENT PROBLEM THAT'S GETTING WORSE AND WORSE IN THE UNITED STATES BECAUSE THERE ARE FREESTANDING CHILDREN'S HOSPITALS LIKE THE ONE I'M AT, BUT THEY TEND TO BE IN LARGE URBAN CENTERS, AND CHILD HEALTHCARE IS DONE IN MOST AREAS IN ADULT HOSPITALS THAT ARE ELIMINATING THEM BECAUSE IT'S BAD BUSINESS BECAUSE THE REIMBURSEMENT RATES ARE, FOR THE SAME PROCEDURE OR THE SAME OCCUPATION OF THE SAME SIZE BED, ARE LOWER FOR CHILDREN BY LIKE A THIRD OR SOMETIMES HALF.

SO THIS IS AN ISSUE, I THINK, FOR CIRM BECAUSE -- AND I DO AGREE THAT CHASING THIS IS GOING TO BE REALLY DIFFICULT FOR THIS ORGANIZATION. BUT GIVEN THE CHARGE OF THIS COMMITTEE, AFFORDABILITY AND ACCESSIBILITY, IT MAY BE THAT IT FOCUSES ON NON-PEDIATRIC AREAS. I KNOW THAT'S STATING -- THAT'S AN ANATHEMA. OF COURSE, WE HAVE TO INCLUDE PEDIATRIC. THERE ARE MANY CHRONIC PEDIATRIC DISEASES THAT ARE GOING TO BE AMENABLE. THERE ALREADY ARE IN TERMS OF CAR-T AND GENE THERAPY. BUT THERE IS THIS ECONOMIC REALITY, AND IT'S THAT, EVEN FOR THE MOST ROBUST, LARGE CHILDREN'S HOSPITALS, THE NUMBERS DON'T COME CLOSE TO ADDING UP, AND THAT'S GOING TO HAVE AN EFFECT ON THAT PEDIATRIC

POPULATION.

SO I'LL STOP THERE, SEAN, BUT I JUST THOUGHT I HAD TO MENTION THAT ONCE AGAIN. I'LL KEEP SENDING YOU ARTICLES TO READ ABOUT THIS.

DR. TURBEVILLE: IT'S VERY IMPORTANT. PLEASE CONTINUE. AND WE MAY HAVE TO THINK ABOUT TWO MODELS, JUST BIFURCATING PEDS FROM ADULTS. THAT MIGHT BE SOMETHING WE MAY WANT TO CONSIDER.

DR. LEVINE: SEAN, I'LL ADD THREE QUICK THOUGHTS TO THAT ONE THAT'S ON THE SURVEY. I THINK IT'S IMPORTANT WE TALK ABOUT PROFITABLE OR BREAKEVEN. IT WOULD BE GOOD TO TEASE THOSE APART BECAUSE I KNOW THE DEDICATED CANCER CENTERS ARE SO BEATEN DOWN, MEANING THEY WERE LOSING $500,000 IN CAR-T FOR SEVERAL YEARS WITH MEDICARE. THEY'RE HAPPY TO BREAK EVEN. BUT WHEN THIS IS A RARE TREATMENT, BREAKING EVEN OCCASIONALLY IS FINE. BUT AS IT BECOMES MORE COMMON, YOU CAN'T RUN A BUSINESS ON THINGS THAT BREAK EVEN.

AND BY THE WAY, BREAKEVEN MEANS YOU BREAK EVEN ON THE DRUG COST. THE SPECIALIZED SERVICES THAT ARE NEEDED TO MANAGE THOSE DRUGS FROM THE PHORESIS TO THE HANDLING AND THE INFUSION, VERY EXPENSIVE. SO I THINK PEOPLE ARE ACTUALLY LOSING MONEY WHEN THEY THINK THEY'RE BREAKING EVEN COMPARED

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TO THE STATUS QUO.

BE THAT AS IT MAY, THINGS ARE BETTER, BUT EVEN SOME OF THE COMMERCIAL PAYORS ARE SAYING WE ARE NOT GOING TO PAY MARGIN ON THESE THINGS. SO IT'S A TOUGH BUSINESS TO BE IN. THAT'S POINT NO. 1.

POINT NO. 2, I WANT TO REINFORCE WHAT YOU SAID ABOUT THE PAYOR IS LOOKING FOR ALTERNATIVES ON PAYMENT MODELS. WE WON'T TEASE THROUGH THOSE TODAY, BUT I WANT TO POINT OUT THAT, OUTSIDE OF GOVERNMENT, EMPLOYERS ARE ALMOST TWO-THIRDS, LIKE 60 PERCENT OF THE REAL PAYOR, EMPLOYERS OF SMALLER POPULATIONS. THEY'RE EXQUISITELY CONCERNED ABOUT A $2 MILLION PAYOUT, $1 MILLION PAYOUT FOR SOMEONE WHO MAY LEAVE THEIR EMPLOYMENT IN A YEAR AND WORK FOR A COMPETITOR. SO THEY ARE LOOKING AND YOU'VE HEARD ABOUT (INAUDIBLE) FUND THIS. WE MAY NEED TO THINK ABOUT -- THINK OUT OF THE BOX. THE SAME WITH THE TOBACCO TAX. DOES THERE NEED TO BE A TAX TO FUND THESE SORT OF TREATMENTS TO MAKE THEM AFFORDABLE IN THE SHORT TERM BECAUSE OUR SYSTEM WAS NOT SET UP FOR 400,000 TO A MILLION DOLLAR TREATMENT. IT JUST WASN'T. SO WE CAN'T RELY ON OLD SCHOOL TO SOLVE THIS PROBLEM.

AND THEN THE THIRD AND LAST COMMENT I WANT TO MAKE IS A CLINICAL COMMENT. AND IT'S A LITTLE

BIT NUANCED, BUT I JUST WANT TO -- WE SHOULD BE CAREFUL AS WE MOVE TO OUTPATIENT. SOME OF THE EASIER TO DELIVER TREATMENTS WILL BE THE ONES THAT ARE OUTPATIENT. WHAT YOU DON'T WANT TO DO -- THINK ABOUT YOURSELF AS A PATIENT FOR A WHILE. YOU'RE WITH A PROVIDER, AND THAT PROVIDER CAN DELIVER ONE-TENTH OF THE THERAPIES THAT ARE OUT THERE IN AN OUTPATIENT SETTING. THEY'RE GOING TO BE INCENTED OR SUBCONSCIOUSLY MOTIVATED TO OFFER YOU THAT ONE TREATMENT WHERE THERE MAY BE NINE OTHER MORE EFFECTIVE TREATMENTS THAT THEY CAN'T DELIVER BECAUSE THEY'RE IN AN INPATIENT CENTER OR THEY'RE NEW OR IN A TRIAL. YOU DON'T WANT TO SET UP A BARRIER WITHIN THE SYSTEM WITH PERVERSE INCENTIVES WHERE YOU GET THE UNINTENDED CONSEQUENCE, YOU'RE GETTING SUBOPTIMAL THERAPY BECAUSE THAT'S WHAT THE DOCTOR CAN DELIVER AS OPPOSED TO HAVING THE ABILITY TO GO A CENTER AND HAVE THE FULL ARRAY OF OPPORTUNITIES.

AND I GET IT. NOT EVERYONE HAS ACCESS TO A CENTER, AND SOME ACCESS IS BETTER THAN NONE; BUT THESE ARE JUST COMPLICATED ISSUES THAT WE NEED TO THINK THROUGH AS THESE THINGS BECOME MORE UBIQUITOUS AND THRE'S MORE CHOICES THAN WE HAVE TODAY.

DR. TURBEVILLE: GOOD POINT. MARIVEL, DO YOU SEE ANY OTHER HANDS RAISED?

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MS. DE LE TORRE: YES. ACTUALLY HARLAN.

DR. LEVINE: THAT WAS JUST ME. I'M DONE.

MS. DE LA TORRE: THAT'S IT THEN.

DR. TURBEVILLE: OKAY. WELL, VERY GOOD. WELL, THIS WAS JUST OUR FIRST, IF YOU WILL, DAY ONE ON THE ACCESS AND AFFORDABILITY, PRESENTING SOME INFORMATION TO YOU. WE ARE A SMALL SHOP RIGHT NOW, BUT I ANTICIPATE THAT WE'LL BE ABLE TO RAMP UP SOME OF THE INTEL WE NEED TO GATHER AND BRING BACK. I DO LOOK, EVEN OFFLINE, PLEASE SEND ME INQUIRIES OR QUESTIONS OR GUIDANCE THAT YOU THINK WE NEED TO FOLLOW UP ON. I DO THINK THIS IS A VERY CHALLENGING AND INTELLECTUALLY STIMULATING PROGRAM. IT IS, OF COURSE, PART OF OUR FIVE-YEAR STRATEGIC PLAN; BUT I'M CONFIDENT THAT WE WILL, AFTER SIX MONTHS, HAVE A ROAD MAP THAT WE CAN PRESENT TO THE ICOC BASED ON THE AAWG'S GUIDANCE. SO THANK YOU FOR EVERYBODY'S COMMENTS.

MR. VICE CHAIRMAN, I THINK THAT'S IT FROM THE QUESTION STANDPOINT. I'LL PUNT IT BACK OVER TO YOU.

CHAIRMAN TORRES: THAT WAS A SILENT THANK-YOU. THANK YOU TO ALL OUR CIRM STAFF AND TO EACH OF YOU WHO PARTICIPATED TODAY, AND THANK YOU AGAIN FOR SPENDING A FRIDAY AFTERNOON, BUT I KNOW

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YOU DO IT FOR THE PATIENTS BECAUSE I KNOW WHERE YOUR HEARTS ARE. SO TAKE CARE AND HAVE A GREAT WEEKEND. WHEREVER WE ARE, LET'S STAY SAFE AND DRY.

(THE MEETING WAS THEN CONCLUDED AT 3:58 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 JANUARY 13, 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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