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: MAY 2, 2023
1 P.M.

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

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I N D E X

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MAY 2, 2023; 1 P.M.

VICE CHAIR BONNEVILLE: THANKS FOR JOINING US TODAY. WE REALLY APPRECIATE ALL THE TIME AND EFFORT YOU'VE MADE TO BE HERE.

I WANTED TO START OFF BY ASKING OUR NEW BOARD CHAIR, DR. IMBASCIANI, TO SAY A FEW WORDS, INTRODUCE HIMSELF. AND SO, VITO.

CHAIRMAN IMBASCIANI: THANK YOU. HI, EVERYONE. GOOD AFTERNOON. SHE CAUGHT ME UNAWARES.

I THINK MANY OF THE BOARD MEMBERS, I THINK, KNOW ME, BUT I'LL JUST SAY HELLO ANYWAY. SO I'M NOW SIX WEEKS ON THE JOB AS THE CHAIR OF THE ICOC, NOMINATED BY THE GOVERNOR AND LIEUTENANT GOVERNOR. AND I SOMETIMES ASK WHY DID THEY DO THAT TO ME. AND I THINK IT'S BECAUSE OF MY EXPERIENCE.

I'VE FOUR GOT CHAPTERS TO MY LIFE. I'VE BEEN IN ACADEMICS, ALTHOUGH MY PH.D. IS IN THE HUMANITIES. I SPENT 27 YEARS IN THE ARMY MEDICAL CORPS WITH FORWARD APPOINTMENTS TO COMBAT ZONES AND TEN YEARS AS THE STATE SURGEON OF THE CALIFORNIA NATIONAL GUARD, ALMOST 30 YEARS AS A CLINICAL SURGEON OF UROLOGY IN THE KAISER PERMANENTE SYSTEM IN LOS ANGELES. STILL CLINICALLY ACTIVE AND DEDICATING MYSELF VOLUNTARILY ONE WEEKEND A MONTH AT

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KERN MEDICAL CENTER BAKERSFIELD COUNTY HOSPITAL TO KEEP MY SURGICAL SKILLS UP. AND EIGHT YEARS SERVING IN THE CABINET OF TWO GOVERNORS, GOVERNOR BROWN AND GOVERNOR NEWSOM.

AND I THINK PROBABLY THE REASON THEY NOMINATED ME IS BECAUSE OF THAT EXPERIENCE WITH LEGISLATIVE, GOVERNMENTAL, REGULATORY, AND FINANCIAL ASPECTS OF GOVERNMENT, INCLUDING PERSONAL CONTACTS WITH ALL OF OUR LEGISLATORS IN SACRAMENTO AND IN WASHINGTON, AND WITH GREAT FAMILIARITY WITH THE BOND PROCESS.

I'M DELIGHTED TO BE HERE AS THE CHAIR OF THE BOARD AND HAVE MY NEW VICE CHAIR HERE AND OUR WONDERFUL PRESIDENT. I THINK WE'VE GOT A TREMENDOUS WORKING TEAM HERE TO LEAD US INTO OUR THIRD DECADE WITH YOUR HELP ALSO, BOARD MEMBERS AND GUESTS. THANK YOU.

VICE CHAIR BONNEVILLE: THANK YOU. MARIANNE, CAN YOU CALL THE ROLL.

MS. DEQUINA-VILLABLANCA: DAN BERNAL. MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MS. DEQUINA-VILLABLANCA: ANN BOYNTON. JAMES DEBENEDETTI.

MR. DE BENEDETTI: HERE.

MS. DEQUINA-VILLABLANCA: DANA DORNSIFE.

MS. DORNSIFE: HERE.

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

DR. HIGGINS: HERE.

MS. DEQUINA-VILLABLANCA: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: HERE.

MS. DEQUINA-VILLABLANCA: HARLAN LEVINE.

DR. LEVINE: HERE.

MS. DEQUINA-VILLABLANCA: PAT LEVITT.

DR. LEVITT: HERE.

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

DR. QADAN: HERE.

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

MS. SHAPIRO: HERE.

MS. DEQUINA-VILLABLANCA: AND THEN DAN BERNAL, CHECKING AGAIN.

MR. BERNAL: PRESENT.

MS. DEQUINA-VILLABLANCA: I THINK I JUST SAW ADRIANA PADILLA.

DR. PADILLA: HERE.

MS. DEQUINA-VILLABLANCA: AND THEN

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MAHESWARI SENTHIL.

DR. SENTHIL: YEAH, HERE.

MS. DEQUINA-VILLABLANCA: WE ARE GOOD. WE HAVE QUORUM.

VICE CHAIR BONNEVILLE: THANK YOU SO MUCH.

TODAY SEAN WILL BE PRESENTING THE FINAL SUBJECT MATTER BEFORE THE ROADMAP COMES TO US NEXT MONTH. AS YOU KNOW, OVER THE COURSE OF THE LAST YEAR, HE AND HIS TEAM HAVE COME TO US, TO THE WORKING GROUP, TO OUTLINE DIFFERENT AREAS AND TACTICS THAT WE MIGHT USE TO HELP PATIENTS IN CALIFORNIA ACCESS OUR TRIALS AND AFFORD THEM. AND THERE ARE DIFFERENT MEASURES TO THAT. AND YOU ARE GOING WALK US THROUGH SOME OF THOSE THINGS TODAY. AND THEN NEXT MONTH WE'LL HAVE A BIGGER PICTURE OF WHAT THE NEXT FEW YEARS HOLDS FOR THIS WORKING GROUP AS WELL AS FOR CIRM IN THESE AREAS. SO, SEAN, TAKE IT AWAY.

DR. TURBEVILLE: THANK YOU, VICE CHAIRMAN AND CHAIRMAN. WELCOME, AAWG MEMBERS. THANK YOU FOR THE OPPORTUNITY TO PRESENT TODAY. I WANT TO FIRST MAKE SURE EVERYBODY CAN SEE MY SLIDES. WONDERFUL.

ALL RIGHT. WE HAVE A VERY INTERESTING DISCUSSION TODAY. AND AS OUR VICE CHAIRMAN MENTIONED, THIS IS THE LAST OF OUR DUE DILIGENCE ON

A NUMBER OF INVESTIGATIONS, IF YOU WILL, THAT WE'VE RESEARCHED WITH RESPECT TO ACCESS AND AFFORDABILITY. SO I'M GOING TO START WITH THIS SLIDE, AND THIS IS OUR ROADMAP TO ACCESS AND AFFORDABILITY STRATEGIES. WE CONTINUE TO WORK FROM THIS AAWG-APPROVED STRATEGY TO FRAME OUR DISCUSSION AROUND ACCESS AND AFFORDABILITY FOR CELL AND GENE THERAPY.

AND AS A REMINDER AND SORT OF PIGGYBACK WHAT OUR VICE CHAIRMAN JUST MENTIONED, THE ROADMAP IS PART OF OUR FIVE-YEAR STRATEGIC PLAN FOR GATHERING THE NECESSARY DATA AND INFORMATION TO SUPPORT REIMBURSEMENT FOR PRODUCTS RESULTING FROM CIRM'S PROGRAMS, INCLUDING DEVELOPING NOVEL HEALTHCARE AND COVERAGE MODELS, WHICH IS THE TOPIC OF TODAY'S DISCUSSION.

SO JUST FOR BACKGROUND, THE WAY WE APPROACHED OUR METHODOLOGY IS THESE, OF COURSE, ARE THE FOUR STRATEGIES THAT WE ALL ALIGN TO WITH RESPECT TO RESEARCH. AND WE TRY TO MARRY TWO APPROACHES HERE. ONE IS WHAT IS THE JOURNEY, THE PATIENT JOURNEY, FOR THE PATIENT THAT'S GOING ALL THE WAY FROM CLINICAL TRIALS, AND WE TALKED ABOUT PATIENT SUPPORT AND OUT-OF-POCKET EXPENSES GOING THROUGH THAT TRIAL, AND ALL THE WAY TO POTENTIAL COMMERCIALIZATION, AND WHAT DOES IT LOOK LIKE FOR A

PATIENT TO ACTUALLY GO INTO THE COMMERCIAL THERAPY.

AND THEN THE OTHER THING WE TRIED TO MARRY THAT WITH IS WHAT DOES IT LOOK LIKE FOR MANY OF THE CELL AND GENE THERAPIES FOR THAT CLINICAL DEVELOPMENT PROGRAM ALL THE WAY TO POSTMARKETING, WHICH WE TALKED ABOUT A COUPLE OF OTHER SESSIONS AGO. SO THE MARRY OF THOSE TWO REALLY GET US IN AREAS WHERE THERE'S PRESSURE POINTS AND AREAS THAT ARE DIFFICULT FOR PATIENTS THAT WE THINK WE CAN IDENTIFY, AND WE HAVE, FOR FUNDING MECHANISMS FOR THE FIVE-YEAR STRATEGIC PLAN.

SO TODAY SPECIFICALLY I DO WANT US TO FOCUS ON COVERAGE ANALYSIS, WHICH IS, TO BE HONEST WITH YOU, HOT OFF THE PRESS. AND THEN I WANT TO FOCUS A LITTLE BIT ON PERFORMANCE-BASED MODELS, WHICH I PRESENTED SOME TIME AGO. THESE ARE THE OUTCOMES-BASED MODELS THAT ARE VERY POPULAR NOW WITH CELL AND GENE THERAPIES. AND WE RECEIVED SOME NEW INFORMATION THAT WAS PUBLISHED LAST MONTH THAT WILL GIVE US SOME INTEL ON HOW PREVALENT THEY ARE AND WHERE THERE'S AN OPPORTUNITY FOR US TO CONSIDER SOME FUNDING MECHANISMS.

SO LET ME FIRST START WITH THIS SLIDE. AND WE ARE LUCKY ENOUGH NOW THAT WE HAVE SOME EXTRA HORSEPOWER, IF YOU WILL, FROM A RESEARCH STANDPOINT.

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AND WE'VE CONSULTED WITH A GROUP THAT CAN PROVIDE DEEP RESEARCH ON PAYER COVERAGES. AND THROUGH THIS PROCESS WE'VE IDENTIFIED A POTENTIAL PROBLEM. AND THAT'S NOT THE BEST WAY TO START THIS TALK, BUT IT IS SOMETHING THAT WE SEE AS SOMETHING THAT SHOULD IMPACT PATIENTS WITH RESPECT TO ACCESS AND AFFORDABILITY.

AND THE PROBLEM STATEMENT IS A LACK OF TRANSPARENCY OF PAYERS' COVERAGE POLICIES FOR CELL AND GENE THERAPIES. SO LET ME WALK YOU THROUGH THIS AND WHERE WE THINK THERE'S A POTENTIAL RECOMMENDATION ON TWO FRONTS. SO WE DO KNOW HISTORICALLY THAT COVERAGE POLICIES ARE LIMITED, WHETHER IT'S SMALL MOLECULE, LARGE MOLECULE, REGARDLESS OF CELL AND GENE THERAPIES. BUT WE ALSO HAVE NOTICED NOW RECENTLY THAT THERE'S BEEN A NUMBER OF POLICIES THAT HAVE RESTRICTED COVERAGE WITH RESPECT TO CELL AND GENE THERAPIES. AS WE ALL KNOW, AGAIN, MANY CELL AND GENE THERAPIES ARE GOING TO REACH THE MARKET WITHIN THE NEXT ONE TO THREE YEARS, MANY WITH HIGH PRICE POINTS. NOW, TO BE FAIR, THOSE PRICE POINTS HAVE BEEN VALIDATED WITH RESPECT TO PHARMACOECONOMICS. SO FROM A COST-EFFECTIVENESS STANDPOINT, THEY ARE WELL RECEIVED BY PAYERS.

BUT ON THE OTHER HAND, PAYERS ARE

DESIGNING NARROW COVERAGE POLICIES FOR CELL AND GENE THERAPY TO REDUCE THEIR FINANCIAL RISK. AND THAT'S NOT SURPRISING. AND THE REASON WHY IT'S NOT SURPRISING IS, ONE, THERE'S AN UPFRONT COST TO MANY OF THESE THERAPIES. AND MANY INSURERS, PARTICULARLY THOSE IN THE MIDDLE, ARE HAVING A VERY DIFFICULT TIME THINKING ABOUT HOW ARE WE GOING TO AFFORD THAT UPFRONT COST RIGHT OUT THE GATE. WE UNDERSTAND, FROM A COST-EFFECTIVE STANDPOINT, DOWN THE ROAD THAT'S GOING TO PAY DIVIDENDS. PARTICULARLY WE ARE TALKING CURING A PATIENT, BUT AT THE SAME TIME PAYERS ARE THINKING ABOUT, WELL, HOW CAN WE RESTRICT SOME OF THESE CELL AND GENE THERAPIES SPECIFICALLY TO THE LABEL. THAT'S IMPORTANT. I'LL TALK ABOUT THAT IN A FEW MINUTES.

SO WHAT WE WERE ABLE TO IDENTIFY, AT LEAST FROM WHAT'S BEEN PUBLISHED RECENTLY, IS A NUMBER OF EXAMPLES OF RESTRICTIVE COVERAGE POLICIES FOR CELL AND GENE THERAPY. AND EVERYTHING THAT'S UP HERE IS LINKED TO A PEER-REVIEWED PUBLICATION. SO MOVING FORWARD, EVERYTHING IN MEDICAL AFFAIRS IS PRESENTED FROM THE AAWG. WE'LL HAVE THE CITATIONS. ALL THE CITATIONS ARE ONLINE. SO IF YOU WANT TO TAKE A LOOK AT ANY OF THIS INFORMATION, IT'S ALL REFERENCED IN THE SLIDE DECK.

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I WON'T GO THROUGH EACH AND EVERY ONE OF THESE, BUT IT DOES SET PRECEDENTS THAT PAYERS DO RESTRICT THE LABEL. AND IT'S NOT SO MUCH WITH RESPECT TO DOSING OR SCHEDULE. IT'S REALLY ABOUT MEDICAL NECESSITY, WHETHER OR NOT THE PATIENT ACTUALLY NEEDS THE ABSOLUTE REQUIREMENT FOR THE GENE THERAPY. AND SO THAT'S WHERE IT GETS A LITTLE BIT SLIPPERY. AND THAT'S WHERE THERE'S AN OPPORTUNITY FOR US TO EXPAND ON THE RESEARCH.

SO JUST REAL QUICKLY, LET ME RUN THROUGH THESE. ZOLGENSMA WAS RESTRICTED A LITTLE BIT WITH RESPECT TO AGE. AND MANY OF YOU KNOW THAT WAS ONE OF THE FIRST GENE THERAPY TRIALS FOR SPINAL MUSCULAR ATROPHY. HEMGENIX JUST RECENTLY FOR HEMOPHILIA B HAS BEEN RESTRICTED TO SOME EXTENT. BLUE CROSS BLUE SHIELD FOR LUXTURNA WHICH WE TALKED ABOUT ACTUALLY IN ANOTHER MEETING YESTERDAY WITH MEDICAL AFFAIRS FOR THE ASSOCIATED RETINAL DYSTROPHY. AND THEN FINALLY -- I'M SURE DR. HARLAN COULD OPINE HERE -- THERE'S BEEN QUITE A BIT OF RESTRICTION ON THE POLICY SIDE WITH RESPECT TO THE IMMUNOTHERAPIES ON THE ONCOLOGY SIDE AS WELL AS THE AUTOIMMUNE SIDE.

SO THE QUESTION IS WHAT CAN WE DO ABOUT IT? AND I'M GOING TO GIVE AN EXAMPLE OF WHAT'S DONE IN INDUSTRY. SO WHEN WE PREPARE FOR A DRUG LAUNCH,

WE KNOW THAT THERE'S GOING TO BE PUSHBACK BY THE PAYERS. AND WE DO A DUE DILIGENCE ON WHICH PAYERS ARE LIKELY TO ALIGN WITH THE FDA LABEL AND WHO IS GOING TO PUSH BACK A LITTLE BIT BASED ON THEY WANT ADDITIONAL INFORMATION, EFFICACY, SAFETY DATA.

NOW, LET ME BACK UP EVEN A LITTLE FURTHER. JUST BECAUSE YOU HAVE FDA AUTHORIZATION, MARKETING AUTHORIZATION, DOESN'T MANDATE PAYER REIMBURSEMENT. IT'S A WHOLE NOTHER ANIMAL. AND SO THIS IS WHERE MARKET ACCESS AND TRADITIONAL MEDICAL AFFAIRS STARTS TO TEE UP WHAT TYPE OF DATA, ADDITIONAL DATA, ARE WE GOING TO NEED TO ADDRESS PAYERS' CONCERNS. SO PAYERS CAN COME BACK AND SAY, WELL, I'D LIKE TO SEE MORE INFORMATION ON CHILDREN LESS THAN TWO YEARS OF AGE. WHAT DO YOU HAVE? AND THIS IS WHERE THE MANUFACTURER, POTENTIALLY THE HOSPITAL WILL SAY, THIS IS WHAT WE HAVE ON FILE. MEANS EVERYTHING THAT WE DID IN OUR CLINICAL TRIAL BUT, MORE IMPORTANTLY, WHAT WE CAN DO AND WHAT HAS BEEN DONE IS DESIGNING NEW TRIALS TO ADDRESS UNANSWERED QUESTIONS FROM PAYERS.

AND WHAT'S BEEN DONE CURRENTLY IS THE REAL-WORLD DATA. SO THIS FITS INTO HOW IMPORTANT THE REAL-WORLD DATA IS TO RESPONDING TO PAYERS' QUESTIONS AND DENIALS TO SOME EXTENT ABOUT

ADDITIONAL DATA.

I'LL CONTINUE TO EMPHASIZE HOW IMPORTANT REAL-WORLD DATA IS THROUGH THIS PRESENTATION. BUT IT IS NOW PRECEDENT THAT REAL-WORLD DATA HAS BEEN USED, NOT ONLY FROM A REGULATORY FILING FOR CONTINUED SUPPLEMENTS OF NDA OR BLA, BUT MORE IMPORTANTLY IT HAS BEEN USED FOR DENIALS BY PAYERS IN THE CELL AND GENE THERAPY SPACE FOR ADDITIONAL EFFICACY AND SAFETY DATA TO SUPPORT THE THERAPY.

SO ONE OF THE THINGS THAT WE WERE THINKING IS THIS IS JUST A SNAPSHOT OF WHAT'S OUT THERE. WE HAVE NOT DONE A FULL REVIEW OF THE COVERAGE ANALYSIS ON PAYERS FOR CELL AND GENE THERAPY. ONE RECOMMENDATION IS THAT WE'D LIKE TO CONVENE A MUCH LARGER ROBUST COVERAGE ANALYSIS FOR PAYER POLICIES FOR CELL AND GENE THERAPY TO REVIEW AND ADVISE ON OPPORTUNITIES CIRM CAN DEPLOY TO HELP CALIFORNIA PATIENTS.

THE OTHER THING THAT I'D LIKE TO STRESS TEST WITH THE AAWG REALLY IS AROUND DENIAL, IF YOU WILL, WHEN WE TALK ABOUT BENEFIT MANAGERS. SO EARLIER IN THESE PRESENTATIONS, WE TALKED ABOUT THE DIFFERENCE BETWEEN PATIENT NAVIGATORS THAT ARE HELPING PATIENTS THROUGH THE CLINICAL TRIAL PROCESS AND THE PATIENT SUPPORT PROGRAM AND HOW THEY MAY BE

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POSITIONED TO COMMUNITY CARE CENTERS OF EXCELLENCE.

THE OTHER THING THAT TAKES PLACE IS THE APPEAL PROCESS. SO IF ANYBODY HERE AT THIS TABLE, CLINICIANS ON THE CALL, THE PATIENT ADVOCACIES, HAVE PROBABLY EXPERIENCED WHAT IT'S LIKE TO HAVE A DENIAL FROM A PAYER, DOES NOT MEET THEIR SPECIFIC REQUIREMENTS FOR A PARTICULAR THERAPY. AND THE PROCESS OF GOING THROUGH THE APPEAL PROCESS CAN BE INCREDIBLY BURDENSOME, NOT ONLY FOR THE CLINIC, AND I KNOW MANY ALPHA CLINICS CERTAINLY HAVE THE EXPERTISE TO DO THIS. BUT WHEN YOU START THINKING ABOUT THE APPEAL PROCESS FOR PATIENTS, THE APPEAL PROCESS FOR THE FAMILY MEMBERS, THE APPEAL PROCESS FOR COMMUNITY DOCS WHO ARE OUT THERE WHO REALLY HAVE A LIMITED STAFF TO BE ABLE TO HANDLE THAT, THAT'S WHERE THINGS REALLY SLOW DOWN.

AND SO THAT'S ANOTHER QUESTION I HAVE FOR THE AAWG AND WHETHER OR NOT THAT'S SOMETHING THAT WE COULD CONSIDER OUT IN THE COMMUNITY CARE CENTERS OF EXCELLENCE. SO, AGAIN, WE HAVE THOSE PATIENT NAVIGATORS THAT ARE HELPING PATIENTS WITH THE CLINICAL TRIALS, BUT SHOULD WE CONSIDER GOING DOWN THIS ROAD WITH RESPECT TO THE ROADMAP BENEFIT MANAGERS WHO HAVE THE EXPERTISE TO BE ABLE TO GET PATIENTS APPROVAL THROUGH INSURANCE IN A TIMELY

MANNER. I CAN SEE THIS BEING A POTENTIAL POSITIVE CERTAINLY FOR PATIENTS OUT THERE IN THE RURAL COMMUNITIES WHO JUST DON'T HAVE THAT TYPE OF SUPPORT.

SO WITH THAT, I'D LIKE TO PAUSE A LITTLE BIT HERE. I KNOW I THREW A LOT AT YOU, BUT I WOULD LIKE TO GET THE INPUT OF THE AAWG, EVEN OUR COLLEAGUES HERE AROUND THE TABLE, OF WHETHER OR NOT WE ARE TRACKING THE RIGHT DIRECTION HERE WITH RESPECT TO LOOKING AT COVERAGE ANALYSIS FOR PAYERS PARTICULARLY IN CELL AND GENE. THERE'S SOME OPINION THAT THIS IS A PATTERN THAT THEY CAN EXPECT MOVING FORWARD, PARTICULARLY WITH HEMOPHILIA B WHICH MANY STUDIES ARE NOW LOOKING AT SPECIFIC MUTATION. AND THERE'S A MUCH LARGER POPULATION THAT DOESN'T HAVE THAT PARTICULAR MUTATION AND WHETHER OR NOT PAYERS ARE ACTUALLY GOING TO COVER THOSE PATIENTS.

SICKLE CELL IS ANOTHER EXAMPLE WHERE THERE'S SPECIFIC MUTATIONS THAT ARE IN THE ORIGINAL TRIAL. ADDITIONAL MUTATIONS WERE NOT. AND THE QUESTION IS IS THAT GENERALIZABLE TO THE REST OF THE SICKLE CELL COMMUNITY? AND SO INDUSTRY AND PATIENT ADVOCACY AND HOSPITALS ARE STARTING TO THINK THROUGH WHAT TYPE OF DATA GENERATION DO WE NEED TO CREATE IN ORDER TO GET READY FOR THAT SORT OF PUSHBACK.

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SO LET ME PAUSE THERE IF IT'S OKAY WITH THE AAWG.

DR. MILLAN: THERE'S A HAND RAISED.

DR. TURBEVILLE: YES. I DON'T SEE THE HANDS, SO I'LL HAVE TO ASK HAVE MARIANNE. FIRST, LET ME GET SOME FEEDBACK, IF WE CAN, AND MAKE SURE THAT WE'RE TRACKING IN THE RIGHT DIRECTION HERE.

MS. DEQUINA-VILLABLANCA: AMMAR QADAN HAS HIS HAND RAISED.

DR. QADAN: YES. THANK YOU. I ALSO WANT TO WELCOME THE NEW CHAIR. WELCOME THE BOARD.

SEAN, THIS IS REALLY GOOD WORK TO START THINKING ABOUT. ONE OF THE THINGS I REMEMBER FEW MONTHS AGO WHEN ABLA SHARED WITH US THE CLINICAL PROGRAM, WHICH I BELIEVE, I DON'T KNOW FOR OTHERS, I BELIEVE WE NEED TO HAVE ANOTHER ROUND OF DISCUSSIONS AROUND THE CLINICAL PROGRAM BECAUSE THAT FAST RUN IS NOT ENOUGH FOR US TO DIGEST THE WORK THAT IS GOING ON, THE DIFFERENT INDICATIONS THAT WE ARE SEEKING, ALL OF THOSE TYPES OF THINGS. SO THERE MIGHT BE A NEED FOR ANOTHER ROUND SO THAT WE CAN GIVE ALSO BETTER FEEDBACK. SO THAT'S ONE THING.

THE SECOND THING, WHEN WE HAD THAT DISCUSSION, I THINK ABLA MENTIONED THAT WE ARE GATHERING, IN FACT, SOME ECONOMIC DATA, HEALTHCARE

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UTILIZATION IN OUR CLINICAL DEVELOPMENT PROGRAM. AND I REMEMBER I WAS ASSURED THAT WE ARE RECRUITING A THIRD PARTY TO DO THAT. SO I DON'T KNOW WHAT TYPE OF DATA IS BEING GATHERED, IF ANY. AND SO MAYBE, IF IT'S NOT DONE, THEN WE NEED TO DO IT.

AND THE THIRD THING IS, GOING BACK TO MY RECOMMENDATION DURING THE LAST MEETING, WE NEED TO HAVE A FULL-TIME HEALTH ECONOMIST ON BOARD TO START LOOKING AT HOW WE WANT TO GATHER THE DATA AND HOW WE WANT TO USE THE DATA. THANK YOU.

DR. TURBEVILLE: THANK YOU, AMMAR. ANY OTHER COMMENTS?

VICE CHAIR BONNEVILLE: JUST REAL QUICKLY, WHEN YOU TALK ABOUT CONVENING COVERAGE ANALYSIS, CAN YOU EXPAND ON WHAT YOU MEAN? DO YOU MEAN YOU ARE GOING TO CALL -- ARE YOU TALKING ABOUT BRINGING PEOPLE TOGETHER? ARE YOU TALKING ABOUT HIRING A CONSULTANT TO GET THIS? ARE WE TALKING ABOUT CONVENING A SUMMIT HERE? LIKE WHAT DO YOU MEAN BY CONVENE COVERAGE ANALYSIS?

DR. TURBEVILLE: I WAS REALLY IMPRESSED WITH WHAT THE TEAM FOUND IN THE LAST COUPLE OF WEEKS. THEY DO HAVE TO DO SOME DIGGING. MY RECOMMENDATION IS TO CONTINUE WHAT WE'RE DOING NOW, LET THE TEAM DIG A LITTLE BIT DEEPER INTO WHAT'S

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BEEN PUBLISHED AND WHAT HASN'T WITH RESPECT TO PAYER POLICIES.

VICE CHAIR BONNEVILLE: OUR TEAM INTERNALLY?

DR. TURBEVILLE: IT COULD BE OUR TEAM AS WELL AS THE CONSULTING TEAM THAT HAS DONE A LOT OF THE RESEARCH. REALLY IMPRESSED WITH THE TEAM. AND JUST FOR BACKGROUND, THIS IS THE ANALYSIS GROUP THAT'S HERE LOCATED IN CALIFORNIA THAT DOES A LOT OF HEOR WORK AND WAS ABLE TO FIND SOME REALLY INTERESTING INFORMATION.

CHAIRMAN IMBASCIANI: I DON'T HAVE A COMPUTER TO RAISE A HAND. JUST FOLLOWING UP ON MARIA'S QUESTION, I'M SURE WE'RE GOING TO BE IMPRESSED WITH OUR INTERNAL. I'D LIKE TO BE IMPRESSED WITH THE COVERAGE PEOPLE. ARE THEY GOING TO BE FORTHCOMING, DO YOU THINK?

DR. TURBEVILLE: IF YOU MEAN GETTING PAYERS IN THE ROOM.

CHAIRMAN IMBASCIANI: YES.

DR. TURBEVILLE: THAT'S A DIFFERENT DELIVERABLE. SO, YES, JUST FOR FEEDBACK, I THINK WHAT WE ARE TALKING ABOUT THERE IS GETTING PAYERS INTO THE ROOM AND START ASKING THEM ABOUT WHAT THEIR CONCERNS ARE WITH RESPECT TO CELL AND GENE THERAPY.

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CHAIRMAN IMBASCIANI: DO THEY HAVE A MODEL THAT THEY MIGHT WANT TO PROPOSE?

DR. TURBEVILLE: YEAH. I KNOW DR. LEVINE AND I TALKED ABOUT THIS, AND THAT'S A GOOD QUESTION. THAT'S A REALLY GOOD QUESTION. I HOPE THEY ARE. I HOPE THEY ARE ABSOLUTELY CANDID SO THEY TELL US HERE'S THE CONCERNS. AND TALKING TO PAYERS MYSELF, WHAT THE CONCERNS ARE, I THINK, WOULD RESONATE WITH THEM, AND THAT IS THAT UPFRONT COST.

I THINK THE OTHER CONCERN IS THE FOLLOW-UP TO SOME EXTENT. WHAT DOES THAT LOOK LIKE? AND THESE CONTRACTS, WHICH I'LL GET INTO AS THE NEXT TOPIC, IS FASCINATING WHAT WE JUST FOUND IN THE LAST TWO WEEKS.

SO I HOPE THE ANSWER IS YES. I THINK WITH THE AAWG'S RECOMMENDATIONS OF WHO WE CAN GET IN THE ROOM, WHETHER IT'S THE BLUE CROSS, KAISERS OF THE WORLD, A SAFE HARBOR FOR THEM TO BE ABLE TO HAVE THAT DISCUSSION WITH US, I THINK YOU CAN GET SOME GOOD INFORMATION.

CHAIRMAN IMBASCIANI: I HAD A SECOND QUESTION, UNRELATED. I'M JUST LOOKING AT THE EXAMPLES OF RESTRICTIVE COVERAGE POLICIES. MY PERSONAL KNOWLEDGE, EXPERIENCE THE LAST 20 YEARS OF HEALTHCARE COVERAGE IN CALIFORNIA, GROUPS OFFERED

WHAT THEY OFFERED UNTIL THE LEGISLATORS SAID NOT ENOUGH, NOT GOOD ENOUGH. SO 20 YEARS AGO ALL HEALTHCARE PROVIDERS IN CALIFORNIA HAD TO OFFER OB-GYN CARE, PERINATAL CARE, WHATEVER. THEN ABOUT 15 YEARS AGO THE MENTAL HEALTH COMMUNITY, THEY GOT A BILL THROUGH THE LEGISLATURE IN CALIFORNIA REQUIRING PARITY IN THE CARE, THE PHYSICAL NEED, YOU ARE EQUALLY OBLIGATED.

SO DO YOU SEE A LEGISLATIVE BOOST FOR THIS? THIS IS PROBABLY PREMATURE, THE QUESTION, BUT MAYBE NOT SO MUCH.

DR. TURBEVILLE: THINKING THROUGH THE ROADMAP, IT'S A FIVE-YEAR STRATEGY. SO I LIKE THAT. THE ANSWER IS YES. WE ARE TALKING ABOUT CELL AND GENE THERAPIES HERE. SO IT IS A LITTLE BIT NICHEY, IF YOU WILL. BUT WHEN YOU TALK ABOUT ONCOLOGY, WHICH IS A BIG BUDGET IMPACT FACTOR FOR PAYERS, CERTAINLY. WE HAVE SORT OF DABBLED IN THE POLICY SIDE IN TERMS OF WHAT WE SUPPORT, WHAT WE CAN CONSIDER PUTTING TOGETHER OURSELVES WITH RESPECT TO THE PROPOSITION. BUT CERTAINLY ALL FOUR, IF WE CAN BE ABLE TO, I WOULDN'T SAY, DRIVE OUR OWN POLICY, BUT CERTAINLY SUPPORT POLICY THAT'S OUT THERE THAT WOULD IMPACT ACCESS AND AFFORDABILITY FOR PATIENTS, PARTICULARLY THE UNDERSERVED AND, MORE IMPORTANTLY,

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THE RURAL COMMUNITIES. THERE IS A RACE RIGHT NOW FOR THE RURAL COMMUNITY, WHETHER IT'S US OR INDUSTRY OR OTHER ORGANIZATIONS. SO THAT'S A GOOD THING TO SEE. BUT WE REALLY NEED TO DO A DEEP DIVE ON OPPORTUNITIES ON POLICY.

MS. DEQUINA-VILLABLANCA: WE HAVE DAVID HIGGINS AND THEN HARLAN LEVINE NEXT.

DR. HIGGINS: THANK YOU. I HAVE A FUNDAMENTAL QUESTION ABOUT WHAT OUR GOALS ARE. IT'S SORT OF ARE WE GOING DEEP OR ARE WE GOING WIDE? AND THE QUESTION SIMPLY IS WOULD WE TRY TO COVER MORE INDICATIONS OR COVER INDICATIONS WITH MORE MONEY? SO THE BENEFITS TO THE PATIENTS, DO THEY COME WITH FUNDING A BROADER RANGE OF INDICATIONS OR MORE MONEY FOR ANY GIVEN INDICATION? I HOPE THAT'S CLEAR.

DR. TURBEVILLE: SO LET ME ANSWER. I THINK THE STRATEGY THAT I'D LIKE TO PROPOSE IS MORE ABOUT DATA GENERATION THAT WE CAN DEVELOP OR AT LEAST THAT WOULD HELP WITH THE PAYERS' QUESTIONS WITH RESPECT TO EFFICACY, ADDITIONAL EFFICACY AND SAFETY DATA. SO WE WOULD WORK DIRECTLY WITH THE PHARMACEUTICAL COMPANIES OR BIOTECH UNLESS THEY ENGAGE WITH US AND GOT A MUTUAL UNDERSTANDING IN TERMS OF WHAT THE SPECIFIC AIM WAS. AND THAT WAS SETTING UP AN INFRASTRUCTURE, WHETHER IT'S COMMUNITY

CARE CENTERS OF EXCELLENCE, THAT REAL-WORLD DATA GENERATION, SO THAT WE CAN PREPARE FOR OUR INVESTMENTS, QUITE FRANKLY. WHAT ARE GOING TO BE SORT OF THE STOP GAPS, IF YOU WILL, FROM PAYERS?

AND CAN WE THINK ABOUT WHAT THOSE CHALLENGES ARE NOW, START COLLECTING THAT DATA, SETTING UP THE TRIALS, AND PREPARE IMMEDIATELY TO RESPOND TO ANY OF THOSE PAYERS THAT HAVE QUESTIONS, ADDITIONAL QUESTIONS, ABOUT EFFICACY AND SAFETY.

AND SO THAT DOES IN ITSELF EXPAND ACCESS TO PATIENTS. I WOULDN'T GET -- ANOTHER LEVEL OF THIS, WE'RE GETTING INTO LAYERS, IS TO THINK ABOUT, WELL, IS THE DATA VALID ENOUGH WHERE WE CAN GO BACK AND HELP ONE OF OUR FUNDEES, FUNDERS, IF YOU WILL, GO BACK TO THE FDA WITH POTENTIAL REAL-WORLD DATA TO CHANGE OR MODIFY OR UPDATE THE SUPPLEMENTAL BLA.

DR. LEVINE: A COUPLE THOUGHTS ON THIS, AND I THINK SOME OF IT GETS INTO THE GRANULARITY OF HOW THESE THINGS GET EXECUTED. SO ONE COMMENT I WOULD MAKE IS, UNLIKE ONCOLOGY WHERE YOU KIND OF GET CANCER AND YOU NEED TO GET TREATMENT RELATIVELY QUICKLY, A LOT OF THESE CONDITIONS ARE CHRONIC CONDITIONS. AND IF YOU HAVE ONE HEALTHPLAN WITH A MORE GENEROUS POLICY THAN ANOTHER ONE, YOU'RE GOING TO GET PEOPLE SWITCHING AT THE END OF THE YEAR. AND

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THEN YOU ARE GOING TO HAVE SORT OF THIS RACE TO THE BOTTOM WHERE NO ONE WANTS TO HAVE BENEFITS FOR THIS.

SO I THINK YOU CAN MAKE A HEALTH POLICY ARGUMENT THAT THERE REALLY NEEDS TO BE PARITY FOR THESE CONDITIONS ACROSS THE HEALTHPLANS. NOT JUST BECAUSE IT'S EASIER FOR THE PROVIDER, BUT OTHERWISE YOU ARE GOING TO GET ANYONE THAT PROVIDES REASONABLE BENEFITS IS GOING TO GET DISPROPORTIONATELY AFFECTED AS PEOPLE MOVE FROM ONE PLAN TO ANOTHER. SO I THINK WE HAVE TO BE REALLY -- I THINK THE LEGISLATIVE APPROACH IS GOING TO BE IMPORTANT HERE. I THINK CONSISTENCY IS IMPORTANT HERE. I THINK YOU CAN ASK YOURSELF WHY WOULD THERE BE DIFFERENT COVERAGE BETWEEN DIFFERENT BENEFITS BETWEEN DIFFERENT PAYERS. THAT'S JUST ONE COMMENT.

THE SECOND COMMENT I WOULD MAKE IS THINK ABOUT -- THE WAY ONCOLOGY IS PAID TODAY IS, PARTICULARLY FOR OUTPATIENT HOSPITAL UNITS AND DOCTOR OFFICES, THEY PURCHASE THE DRUG AND THEN THEY INFUSE THE DRUG AND THEY CHARGE A MARGIN. AND USUALLY THE MARGIN IS A PERCENTAGE OF THE COST OF THE DRUG, WHICH, BY THE WAY, NEVER REALLY MADE ALL THAT MUCH SENSE, BUT THAT'S THE WAY IT'S BEEN DONE. THERE'S A TREND NOW WITH KYMRIAH AND THE OTHER CAR-T'S NOT TO WANT TO PAY A MARGIN BECAUSE THESE

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ARE $400,000 DRUGS. THEY DON'T WANT TO PAY THE 20 PERCENT, 10 PERCENT, 6 PERCENT, WHATEVER IT IS.

AND WHAT'S HAPPENED NOW IS THAT THE PAYERS ARE ARGUING TO PAY ZERO ON TOP OF THE DRUG COST, WHICH IS A DISINCENTIVE FOR THE PROVIDERS TO ACTUALLY BOTHER TO GIVE THE DRUG BECAUSE THEY'RE TAKING UP A BED OR A CHAIR WHERE THEY COULD BE MAKING MARGIN AS OPPOSED TO BREAKING EVEN. SO THIS ISN'T SAID TO BENEFIT PAYERS, BUT I THINK IT'S TO CREATE A MARKET TO BENEFIT PROVIDERS. BUT IF YOU DON'T ALLOW FOR SOME SORT OF MARGIN FOR THE PROVIDER BECAUSE THESE DRUGS ARE, QUOTE, SO EXPENSIVE, YOU'RE NOT GOING TO HAVE ANY PROVIDER WANTING TO GIVE ANY OF THESE DRUGS. I THINK WE WANT TO PROTECT AGAINST ELIMINATING THE MARKET, NO. 2.

AND THEN THE LAST THING I WOULD JUST COMMENT ON, YOU TALKED ABOUT THE VALUE OVER A LIFETIME. FIRST OF ALL, PAYERS DON'T WORRY ABOUT LIFETIME VALUE UNLESS YOU'RE MEDICARE. BUT, SECONDLY, I THINK THE BIGGER CHALLENGE IS GOING TO BE PAYERS LEFT TO THEIR OWN DEVICES. WE'LL HAVE DIFFERENT DEFINED VALUE PROPOSITIONS FOR THINGS THAT WORK FOR NINE MONTHS OR 12 MONTHS AND THEN NEED REPEAT TREATMENT. AND SOME WILL SAY, WELL, THE COST BENEFIT ANALYSIS ISN'T WORTH IT, SO WE'RE NOT GOING

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TO COVER IT AND OTHERS WILL COVER IT. YET ANOTHER REASON WHY WE NEED TO HAVE TO TRY TO TAKE A STRONG STAND AND TRY TO GET CONSISTENT POLICIES ACROSS PAYERS AT LEAST IN CALIFORNIA.

DR. TURBEVILLE: VERY GOOD. THANK YOU.

MR. DE BENEDETTI: ONE THING I THINK YOU SHOULD DO, AND MAYBE YOU'VE DONE THIS ALREADY IN YOUR REVIEW OF THE LAWS, TALK TO THE REGULATORS, DEPARTMENT OF MANAGED HEALTHCARE, AND MAYBE ALSO DEPARTMENT OF INSURANCE TO SEE WHAT THE EXPECTATIONS ARE IN TERMS OF PLANS, WHAT THEY'RE REQUIRED TO COVER. YOU CAN SEE WHAT THE FLOOR IS AND SEE WHAT CARRIERS, IF ANY, ARE ABOVE THAT FLOOR AND MAYBE SOME AREN'T EVEN MEETING THAT FLOOR, AND THE REGULATORS AREN'T AWARE OF THAT.

YOU ALSO HAVE THE ISSUE OF ERISA PLANS, SO SELF-FUNDED OFFERINGS OR PLAN OFFERINGS. EMPLOYERS OFTEN DON'T HAVE THE SAME REGULATIONS OVERSEEING THEM. THEY'RE EXEMPT FROM A LOT OF THEM. SO I DON'T KNOW TO THE EXTENT THAT MIGHT HAVE AN IMPACT ON WHAT HAS TO BE COVERED OR NOT. SOMETHING REGULATORS SHOULD BE ABLE TO TELL YOU ABOUT THOUGH.

DR. TURBEVILLE: THANK YOU, JAMES. THAT'S GOOD.

THIS TEES UP THE NEXT SLIDE. SO PAYER

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MODELS. WE WERE ABLE TO DO A DEEP DIVE ON PAYER MODELS AS MUCH AS WE COULD. NOW, THESE ARE PROPRIETARY, OF COURSE, WITH COMPANIES AND MANUFACTURERS.

WHAT WE'VE IDENTIFIED IS A LACK OF STANDARDS FOR PERFORMANCE-BASED PAYMENT MODELS. AND I PRESENTED ON THIS A COUPLE OF TIMES IN THE PAST. I WON'T GET INTO GREAT GRANULARITY, BUT THERE'S FOUR OF THEM THAT ARE PREVALENT OUT THERE. THE ANNUITY-BASED PROGRAMS, OUTCOMES BASED-PROGRAMS, A HYBRID THEREOF, AND VALUE-BASED WARRANTIES, WHICH JUST A PRESS RELEASE FOR A BIOTECH COMPANY HERE IN THE BAY AREA IS USING FOR THEIR HEMOPHILIA.

SO THESE ARE ABSOLUTELY CRITICAL AGAIN WHEN IT COMES TO REAL-WORLD DATA GENERATION. AND THAT'S PROBABLY THE LAST I'LL SAY ABOUT REAL-WORLD DATA OTHER THAN THE FACT I THINK IT'S PROBABLY GOING TO BE A YEAR ONE STRATEGY WITH RESPECT TO THE ROADMAP. IT IS JUST THAT CRITICAL MOVING FORWARD TO MAKE SURE WE HAVE THE INFRASTRUCTURE, THE METHODOLOGY. AND IT'S NOT SO MUCH ABOUT COLLECTING DATA. IT'S ABOUT COLLECTING GOOD DATA THAT WILL MEET THE PAYERS' REQUIREMENTS AS WELL AS POTENTIALLY THE COMPETENT AUTHORITIES' REQUIREMENTS WHEN IT COMES TO REAL WORLD. ANOTHER TOPIC FOR ANOTHER

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TIME.

I DO WANT TO REPORT ON A SURVEY THAT WAS JUST RECENTLY PUBLISHED OR PRESENTED AT THE ACADEMY OF MANAGED CARE PHARMACY. AND SO THE AMCP IS SORT OF THE THINK TANK WHEN IT COMES TO SETTING GUIDELINES FOR DOSSIERS THAT GO TO PAYERS, PUBLIC PAYERS, CMS, ET CETERA. THEY ARE THE HEAVY HITTERS. THEY SET THE GUIDELINES IN TERMS OF WHAT HAS TO BE IN THAT DOSSIER FOR PAYERS. PAYERS ACCEPT THAT, AND THEN THAT'S HOW THE DISCUSSION PROCEEDS.

SO THIS SURVEY WAS FASCINATING. SO IT WAS DESIGNED TO UNDERSTAND THE U.S. PAYER PERSPECTIVE AND EXPERIENCES WITH INNOVATIVE CELL AND GENE THERAPY CONTRACTS. AND THE SURVEY WAS SENT TO 30 DIFFERENT HEALTHPLANS. THERE WERE PRIVATE. THERE WERE A COUPLE PUBLIC HEALTHPLANS AS WELL.

AND WHAT THEY FOUND WAS THE PAYMENT MODELS ARE MUCH MORE PREVALENT THAN PREVIOUSLY RECOGNIZED AND WELL ACCEPTED BY PAYERS. THE OUTCOMES-BASED PAYMENTS, WHICH I TALKED ABOUT A COUPLE WEEKS AGO, WERE THE MOST COMMON. AND THEY FOUND THAT CLINICAL TRIAL DATA WITH REAL-WORLD EVIDENCE WERE CONSIDERED VALUABLE ENDPOINTS FOR MONITORING PROGRESS AND MEASURE OF THE SUCCESS OF INNOVATIVE CONTRACTS.

SO, AGAIN, THIS ALIGNS WITH OUR SORT OF

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THINKING AND STRATEGY AND ALIGNS, OF COURSE, WITH THE FDA'S GUIDANCE ON THE VALUE OF REAL-WORLD DATA.

SO BASED ON THE SURVEY, THEY THOUGHT THAT IN THE NEXT THREE TO FIVE YEARS, RESPONDENTS INDICATED THAT ONCOLOGY, NEUROLOGY, AND IMMUNOLOGY ARE THE TOP THREE DISEASE STATES OF INTEREST FOR INNOVATIVE CONTRACTS, WHICH, OF COURSE, WE'RE IN ALL THREE OF THOSE DISEASE SPACES.

THE AUTHORS CONCLUDED THAT AS MORE NOVEL CELL AND GENE THERAPIES ENTER THE MARKET, INNOVATIVE CONTRACTS WILL PLAY A CRUCIAL ROLE IN REDUCING COST AND IMPROVING ACCESS. AND SO ONE OF THE RECOMMENDATIONS THAT WE HAVE TO THE AAWG FROM THE TEAM HERE IS THAT WE WOULD LIKE TO EXTEND OUR RESEARCH STANDARDS WITH RESPECT TO INNOVATIVE PERFORMANCE-BASED MODELS. SO WE WOULD LIKE TO TAKE THE LEAD IN IDENTIFYING WHAT THOSE STANDARDS SHOULD BE, AND NOT ONLY FOR THE STATE, AND WE CAN WORK WITH THE ALPHA CLINICS IN DOING THIS, BUT THERE ALSO HAS BEEN A CALL FROM CMS TO DO THE SAME THING WITH STATES. STATES COME TO US, TELL US WHAT ARE THE THREE BEST MODELS THAT WE SHOULD CONSIDER FROM AN OUTCOMES-BASED PERFORMANCE MODEL, AND WOULD CONSIDER THEN PUTTING IT INTO CMS.

AND SO THERE'S SORT OF TWO PLAYS HERE.

ONE, LET'S STRIKE UP MAYBE A STRIKE TEAM, IF YOU WILL, THAT WOULD INTERNALLY BE ABLE TO GIVE US GUIDANCE ON WHAT SHOULD BE IN THOSE PAYER MODELS. AND MORE IMPORTANTLY, GUIDANCE FROM THE TEAM IN TERMS OF HOW WE CAN ENGAGE THE ALPHA CLINICS AND OTHER SUBJECT MATTER EXPERTS THAT WOULD BE ABLE TO HELP US GET TO THAT FINISH LINE.

LET ME OPEN IT UP FOR ADDITIONAL QUESTIONS. DOES THAT MAKE SENSE? ANYTHING I NEED TO CLARIFY, PERHAPS GO A LITTLE BIT MORE DETAIL?

DR. LEVINE: CAN YOU JUST REPEAT THE ASK THAT YOU'RE ASKING US TO RESPOND TO OR OPINE ON?

DR. TURBEVILLE: LET ME MAKE SURE WE ARE TRACKING CORRECTLY HERE. ONE IS WE PLAN TO DO ADDITIONAL RESEARCH ON IDENTIFYING THE STANDARDS FOR INNOVATIVE PERFORMANCE-BASED PAYMENT MODELS. TO COLLECT RELEVANT AND APPROPRIATE OUTCOMES DATA. ADDRESSING OPERATIONAL CHALLENGES IN DATA COLLECTION WHILE MAXIMIZING PATIENT ACCESS AND AFFORDABILITY.

SO REALLY BETWEEN THIS TEAM, DOES THAT RESONATE WITH FOLKS? DOES THAT SEEM LIKE THAT WOULD BE A PART OF THE ROADMAP THAT WE WOULD CONSIDER FOR ACCESS AND AFFORDABILITY? AND DO YOU SEE THAT BEING A WIN-WIN ON MANY LEVELS?

VICE CHAIR BONNEVILLE: AMMAR HAS HIS HAND

RAISED. GO AHEAD, AMMAR.

DR. QADAN: I THINK IT'S THE RIGHT WAY TO GO, SEAN. HOWEVER, I WOULD SUGGEST THAT NOT LOSING TIME AND RESOURCES ON GETTING GENERAL RECOMMENDATIONS AND FOCUS ON OUR CLINICAL PROGRAM AND WHAT IT IS SUPPOSED TO DELIVER, AND THEN USE THAT TO DO THE RESEARCH IN MORE DETAILS. WE ARE RUNNING THE RISK OF GETTING SOME GENERALITIES OUT OF THAT TYPE OF RESEARCH THAT MIGHT NOT BE THAT HELPFUL OR MINIMALLY HELPFUL. THANK YOU.

VICE CHAIR BONNEVILLE: SEAN, I ALSO HAVE A QUESTION. WHEN YOU TALK ABOUT RESEARCHING THE STANDARDS, WHO DO YOU GO TO FOR THAT? LIKE WHERE DO GET THAT INFORMATION? WHO ARE THE PLAYERS INVOLVED?

DR. TURBEVILLE: SO THIS STUFF IS HIGHLY PROPRIETARY. PAYERS DON'T GIVE UP THIS INFORMATION AND CERTAINLY CONSULTING FIRMS DON'T, BUT THEY WILL GIVE YOU GUIDANCE IN TERMS OF WHAT THEY'RE SEEING OUT THERE. AND THE TEAM THAT WE'RE WORKING WITH ACTUALLY DOES CREATE VALUE-BASED CONTRACTS FOR THE INDUSTRY. AND SO THEY CAN GIVE US GUIDANCE IN TERMS OF WHAT DIRECTION WE NEED TO GO IN AND WHAT TYPE OF DATA WE SHOULD CONSIDER COLLECTING.

VICE CHAIR BONNEVILLE: YES, MARIA. DO YOU HAVE A QUESTION?

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DR. MILLAN: THANK YOU. SO I WANTED TO -- MARIA MILLAN. I CAN'T SEE MANY OF YOU BECAUSE I'M IN THE ROOM. BUT A COUPLE OF COMMENTS. AMMAR BROUGHT UP THE HEALTHCARE ECONOMICS RESEARCH EARLIER AND THEN THIS TOPIC. AND SOME OPPORTUNITIES THAT THE AAWG CAN RECOMMEND ARE CREATING FUNDING PROGRAMS THAT MARRY THESE TYPES OF RESEARCH FOR PURPOSE TYPE OF ANALYSIS THAT HAVE CERTAIN STANDARDS TO THEM WITH OUR PROGRAMS.

THERE'S ALSO THE OPPORTUNITY TO FUND ENTITIES THAT COULD DO THIS ON OUR BEHALF FOR OUR PROGRAMS. SO I JUST WANTED TO POINT OUT KIND OF OUR BUSINESS MODELS AS A FUNDING AGENCY AND HOW WE CAN MOVE SOME OF THESE OPPORTUNITIES FORWARD.

REGARDING HEOR DATA THAT'S BEEN GENERATED IN THE PAST, ABLA MAY HAVE MENTIONED THAT SOME OF OUR PROGRAMS DID DO SOME OF THIS ANALYSIS, BUT THEY WERE ESSENTIALLY CUSTOMIZED FOR THE GIVEN TRIAL, DIFFERENT PLATFORMS, ET CETERA. BUT WE DID CONSIDER IT IMPORTANT. SO THEY WERE ALLOWABLE WITHIN OUR CLINICAL PROGRAMS.

ONE COULD ENVISION THAT IT'S NOT ONLY SOMETHING THAT'S ALLOWED, BUT COULD BE AN EXPECTATION THAT WE WOULD HAVE FOR OUR CLINICAL PROGRAMS TO HAVE THE BEST TYPE OF EVIDENCE

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GENERATION, NOT JUST FOR THE SCIENTIFIC AND CLINICAL EVIDENCE GENERATION, BUT IN TERMS OF HEOR AND EVEN REAL-WORLD EVIDENCE AND DATA THAT IS IMPORTANT FOR THIS PROGRAM, NOT ONLY FOR REGULATORY APPROVAL, BUT FOR COVERAGE DECISIONS LATER.

AND I'M NOT AS FAMILIAR AS MANY OF YOU PROBABLY ARE, BUT I HEARD A REALLY GREAT TALK. WE'LL CIRCULATE IT. MARK MCCLELLAN GAVE AN UPDATE ON THE NEW CMS RULES AND THE PRICE REDUCTION ACT AND WHAT THE POTENTIAL IMPLICATIONS ARE. CERTAINLY ONE OF THE ASPECTS IN TERMS OF CMS IS THEIR USE OF REAL-WORLD EVIDENCE IN INFORMING THEIR DECISIONS. SO IT'S EXTREMELY RELEVANT. WE DON'T KNOW WHAT THE IMPACT IS GOING TO BE OF THIS POLICY.

THERE IS A SENSE THAT ACTUALLY IT WILL ACTUALLY BENEFIT THESE RARE, HIGH COST INDICATIONS IN SOME WAY, KIND OF EQUALIZING. I DON'T KNOW IF ANY MEMBERS OF THE AAWG HAVE ANY THOUGHTS ABOUT THAT. BUT IN ANY CASE, I THINK THE IMPORTANT THING IS THE TYPE OF EVIDENCE THAT IS GOING TO BE CONSIDERED IMPORTANT IS SOMETHING THAT'S BEING BROUGHT UP BY SEAN TODAY. THANK YOU SO MUCH.

MS. DEQUINA-VILLABLANCA: JAMES HAS HIS HAND RAISED.

MR. DE BENEDETTI: I'D SAY THIS APPROACH

MAKES A LOT OF SENSE TO A LOT OF PEOPLE, AND I'VE HEARD A LOT OF TALKING ABOUT IT YEARS IN THE PAST. BUT I DON'T KNOW HOW MAY ORGANIZATION HAVE ACTUALLY IMPLEMENTED IT. SO I WOULDN'T BUILD YOUR WHOLE PROGRAM AROUND IT UNTIL YOU DID SOME MORE RESEARCH. DON'T RELY ON IT BEING A SOLUTION TO YOUR PROBLEMS BECAUSE IT IS A LENGTHY, YEARS, IF NOT DECADES LONG APPROACH. AND NOT EVERYONE WANTS TO BUILD THEIR PAYMENTS AROUND SOMETHING LIKE THAT. IT CAN GET KIND OF COMPLICATED AS WELL. MIGHT BE MORE IN THE DOMAIN OF STATES RATHER THAN EMPLOYERS OR CARRIERS OR THINGS LIKE THAT. SO KEEP THAT IN MIND. DON'T PUT TOO MUCH EXPECTATION ON THIS EVEN THOUGH IT SOUNDS LIKE IT MAKES SENSE.

DR. TURBEVILLE: GOOD. THANK YOU.

DR. MILLAN: SORRY. JAMES, THANK YOU SO MUCH. IT'S MARIA MILLAN. CAN YOU CLARIFY DON'T PUT TOO MUCH EMPHASIS ON THIS, ON THE APPROACH OF EVALUATING INNOVATIVE PAYMENT MODELS?

MR. DE BENEDETTI: I THINK IT'S WORTH EXPLORING, BUT I WOULDN'T BUILD YOUR PROGRAM WITH THE ASSUMPTION THAT THAT IS WHAT WILL SOLVE YOUR PROBLEMS BECAUSE YOU MAY NOT BE ABLE TO GET THINGS LIKE THIS TO BE WIDESPREAD LIKE YOU MIGHT LIKE THEM TO IF IT ONLY WORKS FOR ONE OUT OF TEN OR ONE OUT OF

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EVERY HUNDRED CASES THAT YOU BRING UP.

DR. MILLAN: THANK YOU.

MS. DEQUINA-VILLABLANCA: PAT HAS HIS HAND RAISED.

DR. LEVITT: SEAN, ARE THERE DISCUSSIONS IN TERMS OF PUBLIC PAYERS AND WHAT -- THEY CAN'T BE AS COVERT IN TERMS OF INFORMATION, RIGHT, ABOUT HOW THEY'RE THINKING ABOUT STRUCTURING PAYMENTS. MEDI-CAL, FOR EXAMPLE, WHERE A THIRD OF THE CALIFORNIA POPULATION IS COVERED, THAT DEALS WITH THE ACCESSIBILITY ISSUE AS WELL. IS THERE INFORMATION COMING FROM -- ARE THEY INVOLVED IN PLANNING HOW THEY'RE GOING TO DEAL WITH WHAT KIND OF MODEL THEY'RE GOING TO USE?

DR. TURBEVILLE: GOOD QUESTION. AND IT ISN'T OBVIOUSLY IN THE PUBLIC DOMAIN, AND THEY'VE BEEN VERY FORTHRIGHT IN TERMS OF -- I'M SPEAKING FROM CMS AT THIS POINT. THEY'RE VERY PROACTIVE IN REACHING OUT TO STATES TO GET GUIDANCE ON WHAT THEY THINK SHOULD BE THE REQUIREMENTS FOR OUTCOMES-BASED PERFORMANCE MODELS.

WE HAVEN'T ENGAGED WITH THE STATE AT THIS POINT. I CAN'T COMMENT ON WHAT MEDI-CAL IS DOING, BUT THAT'S CERTAINLY ON OUR RADAR. AND MAYBE SENATOR TORRES, PERHAPS, CAN START GETTING THOSE

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INTERACTIONS STARTED.

DR. LEVITT: GOOD. SORRY.

DR. TURBEVILLE: WE HAVEN'T ENGAGED WITH THE STATE AT THIS POINT. I THINK THERE'S A LITTLE BIT OF -- TO BE HONEST WITH YOU, MANY STATES ARE A LITTLE BIT HESITANT IN TERMS OF WHICH DIRECTION THEY NEED TO GO IN RIGHT NOW. I THINK IT'S COMING FROM THE TOP DOWN RATHER THE BOTTOM UP. AND AS MY COMMENT EARLIER IS REALLY ABOUT, HEY, LET'S TAKE IT FROM THE BOTTOM UP AND SEE IF WE CAN DRIVE SOME OF THESE ACTIVITIES WITH THE STATE AS WELL AS ALL THE WAY UP TO CMS.

VICE CHAIR BONNEVILLE: I THINK SOMETHING ELSE TO CONSIDER IS WHAT EXACTLY OUR ASK IS OF THEM. SO WE HAVE TO LOOK AT WHERE OUR PORTFOLIO IS. IS IT ACROSS CELL AND GENE THERAPY THAT WE WANT THE INFORMATION? IS IT SOMETHING SPECIFIC TO ONE OF OUR PROGRAMS? SO I THINK WE HAVE TO CRAFT OUR OWN STRATEGY INTERNALLY FOR WHAT WE WANT FROM THEM IN ORDER TO BE ABLE TO DRIVE THE CONVERSATION.

DR. LEVITT: THERE'S A EUROPEAN AGENCY THAT DOES THIS ACROSS ALL OF EUROPE FOR DIFFERENT COUNTRIES IN TERMS OF DEFINING EXACTLY WHAT THEY MEAN IN TERMS OF THE TYPES OF THERAPIES. SO MAYBE IT'D BE WORTH LOOKING AT. I'M NOT AN EXPERT IN THIS

AREA. I JUST READ THIS STUFF AND SEE WHAT THEY'RE TRYING TO DEFINE AS THERAPIES THAT THEY WOULD PUT UNDER THIS RUBRIC, AND THEN FIGURE OUT HOW THEY'RE GOING TO USE PERFORMANCE TO DETERMINE ACCESSIBILITY. THEY HAVE VERY DIFFERENT PAYER MODELS. SO THAT'S NOT GOING TO BE RELEVANT.

BUT I'M ALSO A LITTLE BIT NERVOUS ABOUT GOING TO OTHER STATES TO GET THEIR INFORMATION ABOUT WHAT THEY'RE DOING BECAUSE CALIFORNIA HAS BEEN PRETTY ENTRENCHED IN THEIR MEDICAID REIMBURSEMENT MODELS FOR, LIKE, DECADES. SO UNLESS THERE'S AN EPIPHANY AND THAT CHANGES, WHICH I DON'T BELIEVE IS GOING TO HAPPEN, IT MAY NOT BE RELEVANT TO GET INFORMATION FROM NEW YORK OR ILLINOIS OR WHEREVER BECAUSE IT JUST DOESN'T APPLY HERE.

DR. TURBEVILLE: OKAY. GOOD.

DR. MILLAN: SEAN, CAN I MAKE A COMMENT?

DR. TURBEVILLE: YEAN, CERTAINLY.

DR. MILLAN: SINCE YOU WERE THE LAST SPEAKER, PAT, I WONDERED IF YOU CAN COMMENT ON WHAT I'M ABOUT TO SAY, WHICH IS THAT AT ONE OF THE PRIOR MEETINGS SEAN HAD AND ABLA CREASEY HAD HIGHLIGHTED THAT CURRENTLY HALF OF OUR PROGRAM IS IN RARE DISEASE. AND WE MAY BE FACING A DIFFERENT SCENARIO FOR RARE DISEASE IN PEDIATRICS THAN WE DO FOR THE

MORE COMMON BROADER INDICATIONS, SUCH AS ONCOLOGY, FOR INSTANCE. SO THAT'S SOMETHING FOR US TO CONSIDER. WHEN WE TALKED TO THOSE FOR THE FIRST ONES OUT THERE MARKETING GENE THERAPIES FOR RARE DISEASE, SUCH AS SPINAL MUSCULAR ATROPHY, ET CETERA, WHAT WE ARE FINDING IS ACTUALLY THEY'RE NOT HAVING AS MUCH OF AN ISSUE GETTING PRIVATE INSURANCE COVERAGE FOR THESE INDICATIONS BECAUSE OF THE NATURE. THESE ARE SEVERELY UNMET MEDICAL NEEDS, FATAL DISORDERS, PEDIATRICS, AND ALL THAT. IT'S MORE OF A STATEMENT MORE THAN ANYTHING.

SO EVEN THIS VALUE BASED -- I THINK THAT WAS A VERY IMPORTANT CAUTIONARY TALE IN TERMS OF HOW MUCH YOU PUT IN TO TAKE STOCK IN TERMS OF ONE-SIZE-FITS-ALL APPROACH TO EVALUATING INNOVATIVE MODELS.

SO THAT DOESN'T MEAN THAT THERE CAN'T BE A UNIFORM, MORE EFFICIENT WAY TO GAIN COVERAGE FOR A RARE DISEASE. IT'S DEFINITELY A NEED. BUT I WANTED TO POINT THAT OUT AND JUST LOVE TO HEAR WHETHER THAT IS, FROM YOUR PERSPECTIVE, THAT THAT IS A FAIR STATEMENT OR YOU HAVE ANY OTHER THOUGHTS ABOUT THAT.

DR. LEVITT: YEAH. I MEAN WHAT WE ARE SUPPORTING NOW IS REALLY FOCUSED ON RARE DISEASES, BUT THAT'S LIKELY TO CHANGE OVER TIME WITH ADVANCES

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IN TECHNOLOGY. RIGHT? SO DISEASES SUCH AS DIABETES ARE NOT RARE. AND CELLULAR THERAPEUTICS IN JUST A RELATIVELY SHORT PERIOD OF TIME HAS ADVANCED PRETTY DRAMATICALLY. AND SO I DON'T KNOW HOW TO ANSWER THAT. I THINK YOU'RE RIGHT IN THE MOMENT, THAT IS CORRECT. AND MAYBE THIS IS FOCUSED ON WHAT WE ARE DEALING WITH IN THE MOMENT. I'M JUST SORT OF THINKING ABOUT HOW THE TREND HAS BEEN OCCURRING IN TERMS OF ALL HEALTHCARE COVERAGE IN THE STATE OF CALIFORNIA FOR A THIRD OF THE POPULATION. AND THE TRENDS ARE NOT GOOD. RIGHT?

AND SO CONTEMPLATING HOW THEY'RE GOING TO DEAL WITH THESE THERAPIES, WHICH ARE UNBELIEVABLY ECONOMIC CHALLENGES, I THINK I DON'T HAVE THE ANSWER TO IT. BUT I THINK YOU ARE CORRECT IN TERMS OF WHAT YOU'RE DESCRIBING IN THE HERE AND NOW.

DR. MILLAN: SO THAT'S IF YOU'RE TALKING ABOUT A PORTFOLIO-BASED APPROACH, I JUST WANTED TO POINT OUT THAT SOME OF THE PORTFOLIO-BASED APPROACH IN THE MOMENT MAY BE RELYING ON THAT KIND OF SPECIALIZED BOUTIQUE TYPE OF COVERAGE. AND THAT AS WE DEVELOP KIND OF THE BROADER -- SEAN HAD PREVIOUSLY MENTIONED TWO TYPES OF KIND OF, I WOULD SAY, DEMONSTRATION CASES OR INDICATIONS THAT ALLOW US TO COVER BOTH. ONE IS THE CANCER INDICATIONS,

THE PART B'S THAT ARE MORE MATURE AND THE RARE DISEASE ONES THAT MAY REQUIRE A DIFFERENT TYPE OF CONSIDERATION. AND THE BOARD MAY SAY THEY'RE BOTH IMPORTANT, OR THE BOARD WITH THE AAWG'S RECOMMENDATION MAY SAY FOCUS ON X OR Y.

SO I JUST POINT IT OUT IN TERMS OF THE TYPE OF DIRECTION THAT THE INTERNAL TEAM WOULD REALLY FIND USEFUL IN TERMS OF DEVELOPING APPROACHES TO BRING MORE INFORMATION TO YOU AND TO THE BOARD. THANK YOU.

DR. LEVITT: I WAS GOING TO SAY, TO FOLLOW UP, ONE OF THE THINGS TO CONSIDER IS THAT WE HAVE A COMMUNICATIONS STRATEGY IN WHICH WE ARE TRYING TO EMPHASIZE THE BREADTH AND DEPTH OF THE IMPACT OF CIRM BROADLY ON THE POPULATION. AND SO I THINK WE HAVE TO BE CAREFUL IN MY MIND ABOUT COMMUNICATING WHERE THE FOCUS IS FOR SOME OF THE DISEASE TARGETS WHICH SCIENTIFICALLY MAKES SENSE, BUT WE DON'T WANT MIXED MESSAGES, RIGHT, THAT IN THE HERE AND NOW WE ARE TALKING ABOUT RARE DISEASES AND THAT PAYER INVOLVEMENT IS GOING TO BE MORE STRAIGHTFORWARD THAN WE THINK.

DR. MILLAN: ABSOLUTELY.

DR. LEVITT: AT THE SAME TIME TALKING ABOUT THE IMPACT ON THE POPULATION OF CALIFORNIA

BROADLY, THOSE ARE MIXED MESSAGES, RIGHT?

DR. MILLAN: YOU'RE ABSOLUTELY RIGHT. THE THING IS THAT THE INITIAL INDICATIONS FOR THE RARE DISEASE, IT'S REALLY A SPRINGBOARD FOR THE LARGER INDICATIONS BECAUSE IT'S A TECHNOLOGY PLATFORM VALIDATION. AND THE IDEA IS THAT THAT WOULD BE BROADER. SO YOU'RE ABSOLUTELY RIGHT. THE TARGET IS BROADER. THANK YOU SO MUCH.

DR. LEVITT: THANKS.

MS. DEQUINA-VILLABLANCA: ADRIANA.

DR. PADILLA: I WAS GOING TO COMMENT THAT COVERAGE HAS ALWAYS BEEN THE BANE OF MY EXISTENCE IN PRIVATE PRACTICE BASICALLY, AND IT'S NOT JUST FOR CELL AND GENE THERAPY. IT'S FOR ANYTHING THAT WE USE IN MEDICINE TODAY, COVERAGE IS HORRIBLE.

WHAT I DO KNOW, THOUGH, IS THAT MEDICARE, USUALLY PART D DOES THEIR OWN CONTRACTING MODELS. AND A LOT OF THE COMMERCIAL INSURANCES KIND OF BASE THEIR COVERAGE TO WHAT MEDICARE DOES. AND HAVE YOU STUDIED THE PART D PART OF MEDICARE TO SEE WHAT KIND OF POLICIES THEY'VE SET FORTH FOR COVERAGE OF RARE DISEASES IN THE OVER 65 POPULATION TO SEE WHAT STANDARDS THEY USE AND WHETHER THAT MIGHT BE HELPFUL IN WORKING WITH THE COMMERCIAL INSURANCES?

DR. TURBEVILLE: THAT'S A GOOD POINT.

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CERTAINLY FAMILIAR WITH PART D IN THE ONCOLOGY SPACE; BUT FOR THE RARE DISEASE SPACE, THAT'S SOMETHING WE'D HAVE TO INVESTIGATE FURTHER. BUT THAT'S A GOOD POINT. THANK YOU.

MS. DEQUINA-VILLABLANCA: ADRIENNE. ADRIENNE, ARE YOU ON MUTE?

MS. SHAPIRO: I'VE GOT A KNOT IN THE PIT OF MY STOMACH BECAUSE WHEN WE ARE LOOKING AT COVERAGE, AND, SAY, FROM MY GROUP, FROM THE SICKLE CELL PERSPECTIVE, THAT WE ARE HAVING REAL TROUBLE ACCESSING WHAT WE NEED FOR COMPARING OURSELVES TO OTHER DISEASES IN THE CHRONIC SPACE, CHRONICALLY ILL SPACE AS WELL AS THE RARE SPACE. SO I JUST WANT TO PUT IT OUT THERE THAT WHEN YOU START TALKING ABOUT LOOKING AT OTHER STATES AND THINGS, I WOULD REALLY LIKE FOR US TO LOOK AT WHAT'S HAPPENING IN CALIFORNIA BECAUSE I THINK WE HAVE AN OPPORTUNITY HERE BECAUSE WE HAVE LIKE EVERYTHING THAT WE NEED. WE HAVE OUR PORTFOLIO, OUR PORTFOLIO THAT'S GOT RARE DISEASES, BUT WE HAVE A LARGE SICKLE CELL POPULATION WHICH IS RARE TO HAVE IN THE RARE SPACE, TO HAVE SUCH A LARGE POPULATION, WHICH WILL ALSO GIVE US SOME INSIGHT TO WHAT HAPPENS THROUGHOUT THE COUNTRY ON LOOKING AT THINGS IN TERMS OF EQUITY AND BIAS.

ALSO, WE HAVE QUITE A BIT OF DOCUMENTATION

ON WHAT HAPPENS WHEN A PERSON, THE META, THE PUBLIC PROGRAM VERSUS PRIVATE BECAUSE WE DO HAVE A PORTION OF OUR POPULATION THAT ACTUALLY IS COVERED BY PRIVATE INSURANCE. AND THE THINGS THAT WE SEE HAPPENING WHEN WE ARE UP AGAINST TRYING TO GET TREATMENT FOR OUR DISEASE COMPARED TO WHAT'S HAPPENING WITH SOMEONE IN TERMS OF BILLING WHO HAS CANCER. SO I LIKE THE IDEA OF US KIND OF DOING A DEEP DIVE AND LOOKING AT REAL-WORLD DATA, BUT IT CAN GET REALLY KNOTTY. THERE CAN BE A LOT OF COMPLICATIONS.

SO I WOULD REALLY LIKE FOR US TO JUST MAYBE KIND OF LOOK AT WHAT WE CAN LOOK AT IN CALIFORNIA AND THEN GO FROM THERE, JUST KIND OF DO OUR -- AS OUR PILOT BECAUSE WE ARE ABOUT TO -- NOBODY EXPECTED TO HAVE A CURE FOR SICKLE GOING AS QUICKLY AS IT HAS. AND IT'S HERE. WE ALL KNOW THAT THIS IS JUST THE BEGINNING. AND THIS IS A TECHNOLOGY-BASED THING AND WE KNOW WHAT HAPPENS WHEN THAT HAPPENS. PEOPLE COME OUT -- THE FIRST OUT THE GATE JUST START AT THE STANDARD AND PROVE IT CAN BE DONE. AND THEN EVERYBODY ELSE STARTS WHERE THEY ARE AND IMPROVES. AND SO IT'S GOING TO GO REALLY, REALLY FAST. I JUST DON'T WANT US GETTING MIRED BECAUSE I BELIEVE THAT I HAVE SEEN THE EFFECT THAT

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THE POLICIES FOLLOWED BY CIRM HAVE MADE IN OTHER STATES. THE REQUIREMENTS THEY HAVE FOR FUNDING, THE QUALITY OF RESEARCH IN THE LAST SEVEN YEARS BY BEING REALLY INVOLVED, I HAVE SEEN THE IMPACT.

SO I THINK WE HAVE, I'M GOING TO SAY, RESPONSIBILITY TO DO THIS IN A WAY WHERE WE ARE COVERING THINGS HONESTLY AND NOT LIKE THEY'VE BEEN DONE BEFORE. AND I THINK THIS IS THE OPPORTUNITY TO REALLY HAVE THAT REAL-LIFE, REAL DATA, AND REAL DATA, NOT JUST -- HOW CAN I PUT THIS? -- MEANING THAT WE ARE REALLY GOING TO BE TOUCHING AND GETTING THE DATA THAT'S HELD BACK, RIGHT, AND HAVING THE HEALTH ECONOMIST HELP US UNDERSTAND BECAUSE THERE'S A LOT THAT GOES ON THAT HAS TO BE EXPLAINED TO LAYMEN ABOUT THAT. BUT I FEEL LIKE IF WE'RE JUST LOOKING AT CALIFORNIA -- I'M BEING REDUNDANT. I'M SORRY. I WOULD LIKE TO SEE US DO THAT BECAUSE WE HAVE IN THAT SENSE A CONTROLLED, KNOWN ENVIRONMENT FROM WHICH WE CAN THEN MAKE HYPOTHESIS AND DOCUMENT AND SHARE THAT INFORMATION.

DR. TURBEVILLE: LET ME CLARIFY SOMETHING. THANK YOU FOR THAT. THAT WAS INCREDIBLY HELPFUL.

WHEN I SPOKE ABOUT OTHER STATES, I DON'T WANT -- I DIDN'T WANT US TO DECIDE THAT WE WERE GOING TO GO LOOK AT WHAT OTHER STATES ARE DOING. I

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THINK MY POINT WAS CMS IS ASKING FOR OTHER STATES, INCLUDING US, GUIDANCE IN TERMS OF WHAT THOSE MODELS LOOK LIKE. JUST A CLARITY.

MS. SHAPIRO: I UNDERSTOOD THAT. WHEN WE START DOING THAT, LOOKING BACKWARDS, NOT BACKWARDS. I DON'T MEAN BACKWARDS. I JUST KNOW HOW FAST THIS IS GOING TO GO. I KNOW THAT I'M LOSING, MY COMMUNITY IS LOSING PEOPLE WHO COULD REALLY BENEFIT FROM THIS TECHNOLOGY. AND SO WE CAN ANSWER THESE QUESTIONS AND GET AHEAD OF THAT CURVE I SEE OF PEOPLE BECOMING UNELIGIBLE, THAT THAT WOULD BE REALLY GREAT. SO I GUESS THAT'S WHAT I'M SAYING. AND WE JUST FOCUS IT AND THEN SHORTER ITERATIONS. DOES THAT MAKE SENSE? AM I --

DR. TURBEVILLE: CERTAINLY. ABSOLUTELY. MAYBE FOCUS JUST ON A SPECIFIC THERAPEUTIC SPACE AND TAKE BABY STEPS WITH RESPECT TO PHASES IN TERMS OF HOW WE APPROACH. THIS IS PART OF THE ROADMAP, TO BE HONEST WITH YOU. SO WE WILL CONSIDER ADDITIONAL RESEARCH. THERE'S A LOT OF UNANSWERED QUESTIONS OUT THERE.

ONE OF THE CHALLENGING AND UNIQUE THINGS ABOUT CELL AND GENE THERAPY IS EVERYTHING IS MOVING SIMULTANEOUSLY. THE PAYER SPACE, THE THERAPIES, HOW DRUGS ARE BEING MANUFACTURED AND DISTRIBUTED, AND WE

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ARE TRYING, I THINK EVERYBODY IS TRYING TO COMPARTMENTALIZE EACH SECTION TO SEE WHERE THERE ARE PLACES THAT WE MIGHT BE ABLE TO HAVE IMPACT ON PATIENTS WITH RESPECT TO ACCESS.

DR. LEVINE: IT'S COME UP --

VICE CHAIR BONNEVILLE: ONE THING I JUST WANTED TO CIRCLE BACK TO IS SOMETHING THAT AMMAR BROUGHT UP AT THE BEGINNING OF THE MEETING WAS UNDERSTANDING WHERE OUR PORTFOLIO IS AND USING THAT AS A BASIS OF SORT THE DIRECTION WE TAKE CONSIDERING OUR WORK IS IN SUPPORT OF THE WORK THAT CIRM DOES AND FUNDS. AND SO REALLY BEING ABLE TO TAKE A LOOK AT THE PORTFOLIO, UNDERSTAND WHERE WE ARE IN RESPECT TO THE DIFFERENT CLINICAL TRIALS WE ARE FUNDING, AND UNDERSTANDING WHAT THEY NEED IN ORDER TO HAVE ACCESS AND AFFORDABILITY FOR THE PATIENTS IN THEIR TRIALS. AND THEN THE PATIENTS IN CALIFORNIA, I THINK, WOULD BE A REALLY GREAT PLACE TO START IF WE COULD GET SOME MOMENTUM THERE.

DR. MILLAN: I KNOW HARLAN WANTS TO SAY SOMETHING ON THAT. SO I WANT TO JUST RAISE KIND OF THE HOW-TOS IN DOING THAT. RATHER THAN CONTINUING TO HAVE PRESENTATIONS BY OUR TEAM, HOW DO YOU REALLY LOOK AT THE PORTFOLIO. SO FOR SICKLE CELL, FOR INSTANCE, THERE ARE TWO PROGRAMS, THERE ARE TWO GENE

THERAPIES THAT ARE ON THE VERGE, THAT ARE DUE TO BE APPROVED THIS YEAR. THEY'RE NOT CIRM PROGRAMS. ONE IS CRISPR AND ONE IS FROM BLUEBIRD BIO. SO THE QUESTION IS --

VICE CHAIR BONNEVILLE: BUT WE ARE FUNDING A SICKLE CELL TRIAL.

DR. MILLAN: WE ARE FUNDING SICKLE CELL, BUT I WANTED TO JUST MAKE CLEAR IT'S NOT NECESSARILY THE PORTFOLIO, BUT IT'S THE INDICATIONS THAT ARE COVERED BY OUR PORTFOLIO. I WANTED TO MAKE THAT DISTINCTION, THAT IT'S NOT A PER-PROJECT BASIS. IT'S A SCOPE BASED TYPE OF ANALYSIS. SO I JUST WANTED TO HIGHLIGHT THAT BECAUSE, IF WE ARE VERY LITERAL ABOUT IT, WE MAY NOT BE DOING WHAT ADRIENNE IS ASKING, WHICH IS LOOKING AT HOW THESE CAN BENEFIT PATIENTS, POTENTIAL CURATIVE THERAPIES THAT ARE AT THIS POINT POTENTIALLY APPROVED. SO THAT'S -- ANYWAY, HARLAN, I'M SORRY TO INTERRUPT.

DR. LEVINE: NOT AT ALL. AT THIS POINT IT'S A LITTLE OFF TOPIC, BUT I JUST THINK WE SHOULD ALSO LOOK BACK TO WHEN CAR-T WAS FIRST RELEASED, THAT CMS APPROVED THE TREATMENT, BUT DID NOT APPROVE PAYMENT. SO PRIVATE PAYERS WERE PAYING FOR IT; CMS WAS NOT. I THINK THERE ARE STILL THINGS WHERE MEDICAID IS NOT PAYING FOR GENOMIC THINGS. I THINK

WE OUGHT TO JUST KIND OF LOOK AT THAT AND MAKE SURE WE'RE NOT IN A POSITION, WE DON'T FIND OURSELVES IN A POSITION AGAIN WHERE THERE'S A DISCONNECT BETWEEN WHAT THE GOVERNMENT IS PAYING FOR AND THE PAYERS ARE PAYING FOR. AND IF SO, WE SHOULD BRING IT TO LIGHT FASTER SO THERE'S NOT THAT GAP THAT WE ARE HEARING, WE'RE CONCERNED ABOUT, PARTICULARLY IN THE MEDI-CAL POPULATION.

DR. TURBEVILLE: GOOD POINT. OKAY. WELL, LIVELY DISCUSSION. SO THANK YOU FOR ALL THE COMMENTS AND FEEDBACK. IT'S VERY HELPFUL.

SO AT THIS POINT, THAT WAS THE LAST OF OUR PRESENTATIONS WITH RESPECT TO THE DUE DILIGENCE ON THE RESEARCH FOR THE ROADMAP. JUST AS A QUICK REFRESHER, WE TALKED ABOUT PATIENT SUPPORT SERVICES, THE NEW PAYER MODELS. AT ONE POINT WE TALKED ABOUT INPATIENT/OUTPATIENT SETTING, PARTICULARLY IN THE CAR-T SPACE. WE DID BRING UP A NUMBER OF OPPORTUNITIES IN STATE AND FEDERAL POLICY. WE OBVIOUSLY HAVE A LOT OF OPPORTUNITY WITH THE ALPHA CLINICS AND THE COMMUNITY CARE CENTERS OF EXCELLENCE AS PART OF THE ROADMAP. THE REAL-WORLD DATA, HEALTH ECON, POSTMARKETING REQUIREMENTS, AND THEN, OF COURSE, TODAY A LITTLE BIT OF COVERAGE ANALYSIS AND INSURANCE RESTRICTIONS THAT WE'RE STARTING TO SEE.

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SO THE NEXT PRESENTATION, WHICH WILL BE IN FOUR WEEKS, WILL BE OUR ROADMAP AND RECOMMENDATIONS. WE'RE LOOKING AT A POTENTIALLY FIVE-YEAR PLAN. WE MAY EXCEED IT OR EXTEND IT TO SEVEN YEARS DEPENDING ON WHAT'S AVAILABLE IN TERMS OF RESEARCH FOR SOME OF THE LATE STAGE INVESTMENTS OR FUNDING OPPORTUNITIES.

SO REALLY LOOKING TO PRESENTING THAT TO THE AAWG IN THE NEXT FOUR WEEKS. AND SO I WANT TO, VICE CHAIRMAN, HAND IT OVER TO YOU AND SEE IF THERE'S ANY ADDITIONAL QUESTIONS OR COMMENTS.

VICE CHAIR BONNEVILLE: ARE THERE ANY COMMENTS FROM THE GROUP? NO HANDS RAISED THAT I SEE. SO THANK YOU, EVERYONE. THANK YOU, SEAN, FOR THIS PRESENTATION. IT WAS GREAT. THANKS TO EVERYONE. WE WILL SEE YOU IN ABOUT A MONTH. IF YOU HAVE ANY QUESTIONS IN THE MEANTIME, PLEASE DO REACH OUT TO SEAN OR TO MYSELF. IF YOU HAVE QUESTIONS ABOUT THE DIRECTION THIS IS TAKING, ANY INPUT THAT YOU MIGHT WANT TO GIVE, IT'S REALLY IMPORTANT TO HEAR FROM YOU, SO PLEASE FEEL FREE TO CALL OR EMAIL. THANK YOU.

DR. TURBEVILLE: THANK YOU, EVERYBODY.

(THE MEETING WAS THEN CONCLUDED AT 2:06 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 MAY 2, 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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