BEFORE THE
IP TASK FORCE
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: THE GLADSTONE INSTITUTE
1650 OWENS STREET
ROOMS C & D
SAN FRANCISCO, CALIFORNIA

DATE: WEDNESDAY, MARCH 29, 2006
8 A.M.

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

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I N D E X

SAN FRANCISCO, CALIFORNIA; WEDNESDAY, MARCH 29, 2006

CHAIRMAN PENHOET: THEN WE'LL GET STARTED. WE HAVE A LONG AGENDA TODAY, LOTS OF INTERESTING PRESENTATIONS TO CONTEMPLATE AND MOVE ALONG. I GUESS WE HAVE SOME REMOTE SITES. MELISSA, WILL YOU DO YOUR NORMAL THING.

MS. KING: I CAN CALL THE ROLL, YES. OKAY. CALLING THE ROLL FROM SAN FRANCISCO. ED PENHOET.

CHAIRMAN PENHOET: HERE.

MS. KING: SUSAN BRYANT.

DR. BRYANT: HERE.

MS. KING: MICHAEL GOLDBERG. SHERRY LANSING.

UNIDENTIFIED SPEAKER: SHERRY LANSING IS NOT HERE IN LOS ANGELES, BUT THE SITE IS OPEN.

MS. KING: PHIL PIZZO.

DR. PIZZO: HERE.

MS. KING: FRANCISCO PRIETO. JOHN REED. JEFF SHEEHY. OS STEWARD.

DR. STEWARD: HERE.

MS. KING: JANET WRIGHT.

CHAIRMAN PENHOET: WELL, WE'RE FAR SHORT OF A QUORUM UNFORTUNATELY, BUT WE DON'T HAVE ANY ACTION ITEMS ON TODAY'S AGENDA. SO THOSE OF US WHO ARE HERE WILL PROCEED TO HEAR THE PRESENTATIONS, DIGEST THEM, AND BEGIN

TO ARTICULATE AN INTELLECTUAL PROPERTY POLICY FOR THE VARIOUS FORMS OF FINANCIAL SUPPORT THAT WE MIGHT GIVE TO FOR-PROFIT ORGANIZATIONS.

AS YOU ALL KNOW, IF YOU'VE BEEN FOLLOWING THIS, WE HAVE A PROPOSAL FOR AN INTELLECTUAL PROPERTY POLICY FOR GRANTS TO NONPROFIT ORGANIZATIONS. THAT POLICY WAS APPROVED BY THE ICOC AT ITS LAST MEETING AND IS NOW BEGINNING TO WEND ITS WAY THROUGH THE SO-CALLED APA PROCESS TOWARDS BECOMING REGULATIONS. BUT PROP 71 CLEARLY ANTICIPATED THAT IN ADDITION TO MAKING GRANTS TO NONPROFIT ORGANIZATIONS, THAT THE CIRM MAY ALSO GIVE GRANTS TO FOR-PROFIT ORGANIZATIONS.

SO THIS NEXT SERIOUS OF MEETINGS THAT WE WILL HOLD ARE DESIGNED TO REALLY, FIRST OF ALL, SURVEY THE FIELD, HEAR FROM PEOPLE WHO ARE EXPERTS IN THE FIELD WHO HAVE EITHER GIVEN OR RECEIVED FROM THE PRIVATE SECTOR, GIVEN GRANTS TO THE PRIVATE SECTOR OR FROM THE PRIVATE SECTOR HAVING RECEIVED GRANTS, AND THERE ARE SOME SIGNIFICANT DIFFERENCES BETWEEN GRANTS, CONTRACTS, OR LOANS THAT WOULD BE MADE TO PRIVATE ORGANIZATIONS AS OPPOSED TO THE FUNDING THAT WE ANTICIPATE FOR THE NOT-FOR-PROFITS.

SOME OF THE DIFFERENCES HERE ARE, FIRST OF ALL, IN GENERAL, THE NONPROFIT INSTITUTIONS DO NOT THEMSELVES DEVELOP AND COMMERCIALIZE PRODUCTS. THEY'RE IN THE

BUSINESS OF DOING BASIC RESEARCH, DOING CLINICAL RESEARCH, RESEARCH OF VARIOUS DIFFERENT KINDS; BUT AT THE END OF THE DAY, IN ORDER FOR THE FRUITS OF THEIR LABOR TO BECOME ESSENTIALLY INCULCATED INTO THE HEALTHCARE SYSTEM, SOME COMMERCIAL ORGANIZATION HAS TO TAKE RESPONSIBILITY FOR THE FINAL DEVELOPMENT, MANUFACTURE, AND SALE OF THE PRODUCTS.

SO NONPROFIT GRANTEES, IN GENERAL, LICENSE THEIR TECHNOLOGY IF IT'S VALUABLE TO FOR-PROFIT ENTITIES. AND WE DID ANTICIPATE IN OUR IP POLICY A VARIETY OF DIFFERENT TERMS THAT THE LICENSEES OF THE NONPROFITS WOULD BE OBLIGATED TO MEET IF THEY TAKE A LICENSE TO TECHNOLOGY GENERATED WITH FUNDS FROM THE CIRM. SO THAT'S THE FIRST ONE.

SECOND POINT IS THERE IS -- THERE ARE LIKELY TO BE SEVERAL DIFFERENT KINDS OF FINANCIAL ESSENTIALLY AWARDS TO FOR-PROFIT COMPANIES. WE COULD IN SOME CIRCUMSTANCES PUT OUT CONTRACTS IF WE SIMPLY WANTED THE PRIVATE SECTOR TO, FOR EXAMPLE, MANUFACTURE A SET OF MONOCLONAL ANTIBODIES WHICH COULD BE USED AS REAGENTS BY ALL PARTICIPANTS IN CIRM. AND WE COULD CONTRACT WITH A PRIVATE ENTITY TO BE A PLACE WHERE PEOPLE STORED CELLS, MASTER CELL BANKS AND THINGS LIKE THAT. THERE ARE A NUMBER OF DIFFERENT WAYS THAT A CONTRACT COULD BE GIVEN TO A PRIVATE ENTITY. GRANTS COULD ALSO BE GIVEN TO

PRIVATE ENTITIES TO DO MANY OF SAME KINDS OF THINGS WE WOULD EXPECT TO BE DONE BY PEOPLE IN THE NOT-FOR-PROFIT SECTOR.

AND THEN FINALLY, IT WOULD BE ENTIRELY POSSIBLE FOR US TO PROVIDE LOANS TO COMPANIES WHO ARE DEVELOPING PRODUCTS IN THIS AREA. SO THERE'S, I THINK, QUITE A DIFFERENT MIX OF FUNDING OPPORTUNITIES FOR US WHEN WE THINK ABOUT FUNDING THE PRIVATE SECTOR THAN THERE WOULD BE WHEN WE FUND THE NOT-FOR-PROFIT SECTOR.

AS A RESULT, WE THINK THAT THE NEW JERSEY MODEL, WHICH JUST PICKED A SINGLE ROYALTY, IF YOU GET MONEY FROM THE STATE OF NEW JERSEY AND YOU'RE A COMPANY IN NEW JERSEY AND YOU DEVELOP A PRODUCT, NEW JERSEY HAS SAID YOU WILL PAY BACK A 1-PERCENT ROYALTY ON ANY PRODUCTS THAT GET DEVELOPED AS A RESULT. WE THINK THAT'S A SIMPLE SOLUTION AND ATTRACTIVE FOR THAT REASON. ON THE OTHER HAND, PROBABLY DOESN'T REFLECT THE RICHNESS OF WHAT MIGHT COME OUT OF THIS IN TERMS OF DIFFERENT WAYS OF MOVING STEM CELL RESEARCH ALONG BY FUNDING ALL THESE DIFFERENT KINDS OF ASPECTS OF INTELLECTUAL PROPERTY DEVELOPMENT IN COMPANIES.

SO WE THINK A SIMPLE PERCENTAGE RETURN IS PROBABLY NOT REFLECTIVE OF THE REALITY AND THE RICHNESS OF THE FUNDING POSSIBILITIES THAT WE HAVE. SO THAT'S WHY WE'RE GOING THROUGH ALL THIS EFFORT TO LOOK HARDER AT HOW

THIS MIGHT WORK.

AND THEN FINALLY, WE WOULD OURSELVES HAVE TO TRACK AND MONITOR THE PROGRESS OF COMPANIES THAT WE GIVE RESEARCH OR OTHER GRANTS, CONTRACTS TO IN ORDER TO KEEP TRACK OF WHAT'S HAPPENED WITH CIRM MONEY. I MIGHT ADD THAT THIS IS A VERY IMPORTANT DISTINCTION FOR CIRM FROM AN OPERATIONAL POINT OF VIEW BECAUSE WE HAVE EFFECTIVELY OUTSOURCED THE LICENSING OBLIGATION TO THE UNIVERSITY WHEN -- WE HAVE EFFECTIVELY OUTSOURCED THE JOB OF LICENSING TECHNOLOGY ON OUR BEHALF TO THE NONPROFITS IN OUR IP POLICY FOR NOT-FOR-PROFITS. WHEN IT COMES TO COMPANIES, THERE'S NOBODY FOR US TO OUTSOURCE IT TO. CIRM WILL HAVE TO BUILD A STAFF, HIRE PEOPLE TO ACTUALLY MANAGE THE GRANTS ITSELF TO THE FOR-PROFIT ENTITY. SO IN THIS CASE WE HAVE TO DO THAT ON OUR OWN AT CIRM.

AND THAT, FRANKLY, REPRESENTS A CHALLENGE GIVEN THE RATHER MODEST OVERHEAD THAT COMES FROM THIS. AS A REMINDER TO PEOPLE ABOUT WHAT HAPPENS IN INNOVATION FROM BASIC RESEARCH TO COMMERCIALIZATION, BASIC RESEARCH IS -- GOOD MORNING, JEFF -- BASIC RESEARCH IS OBVIOUSLY WHAT IT SAYS. IT'S RESEARCH, FUNDAMENTAL RESEARCH, THAT SORT OF UNDERPINS THE ENTIRE FIELD AND IS APPLICABLE TO MANY DIFFERENT PRODUCTS. BASIC RESEARCH TURNS INTO APPLIED RESEARCH WHEN PEOPLE CHOOSE A SPECIFIC GOAL THAT THEY WANT TO ACHIEVE, SPECIFIC THERAPY, ETC., AND APPLY

THEMSELVES TO THAT.

DEVELOPMENT REFERS TO THE WHOLE SET OF ACTIVITIES THAT ARE REQUIRED WHICH WILL EVENTUALLY LEAD TO APPROVAL OF A PRODUCT BY THE FDA. SO IT STARTS WITH STUDIES IN ANIMALS AND LEADS TO PRECLINICAL STUDIES OF VARIOUS DIFFERENT KINDS AND ULTIMATELY HUMAN CLINICAL TRIALS. AND IF THOSE HUMAN CLINICAL TRIALS ARE SUCCESSFUL, FINALLY, IN THIS COUNTRY FDA APPROVAL, AND AFTER THAT COMMERCIALIZATION. IT'S CONCEIVABLE, IN FACT QUITE LIKELY, THAT CIRM COULD FUND PROJECTS ANYWHERE IN THIS CONTINUUM EXCEPT PROBABLY IN THE COMMERCIALIZATION SECTOR, BUT WE COULD FUND BASIC RESEARCH IN COMPANIES, WE COULD FUND APPLIED RESEARCH IN COMPANIES, WE COULD FUND CLINICAL DEVELOPMENT IN COMPANIES. ALL OF THESE ARE POSSIBILITIES.

AND I THINK IF IN LOOKING AT ULTIMATELY WHETHER TO PUT MONEY INTO PRIVATE COMPANIES OR INTO THE NOT-FOR-PROFIT SECTOR, THE GUIDING PRINCIPLE IS LIKELY TO BE WHERE WILL THE STATE GET THE BEST OVERALL RETURN FOR ITS MONEY, MOVING THE SCIENCE ALONG FASTEST, YOU KNOW, IN GENERAL, LOOKING AT THE OVERALL PICTURE, SO IT'S NOT A SIMPLE MATTER TO FIGURE OUT HOW WE SHOULD PROCEED.

AS I THINK I INDICATED BEFORE, THE FOR-PROFIT POLICY WILL NEED FLEXIBILITY BECAUSE IT'S VERY HARD TO ANTICIPATE TODAY ALL THE DIFFERENT CIRCUMSTANCES UNDER

WHICH WE MAY WANT TO FUND THE FOR-PROFIT SECTOR IN ORDER TO MOVE THIS TECHNOLOGY ALONG. THERE WILL BE SUBSTANTIAL DIVERSITY, AND WE HAVE TO THINK ABOUT ALL THESE POSSIBILITIES. WE HAVE TO UNDERSTAND THE REALITIES AT THE GRANTOR AND GRANTEE LEVELS IN ORDER TO UNDERSTAND THE POLICY EFFECTS AT THE STATE LEVEL. SO THIS IS, I THINK, QUITE A CHALLENGE.

AND IN CONTRAST, NOW I'M JUST SPEAKING A PERSONAL OPINION OF MINE, IN CONTRAST TO THE, I THINK, PRETTY TIGHT LANGUAGE WE HAVE IN THE NOT-FOR-PROFIT IP POLICY, BECAUSE THESE POLICIES WILL BE PUT IN PLACE BY A STATE AGENCY, CIRM, AND THERE ARE MANY DIFFERENT WAYS TO DO THIS, I THINK IT MAY BE THAT OUR POLICY HERE WILL BE PERHAPS MORE CONCEPTUAL AND MORE BASED ON PRINCIPLES THAN IT IS ON VERY PRECISE DETAILS ABOUT STUFF. WE'LL SEE HOW THAT EMERGES.

THERE ARE A NUMBER OF WAYS THAT THE FEDERAL GOVERNMENT HAS BEEN ENGAGED IN THAT. AND IN A MOMENT WE'RE GOING TO HEAR FROM GREG MILMAN, WHO IS ONE OF NIH'S MOST ACTIVE PEOPLE IN THIS WHOLE AREA AND WITH A LOT OF EXPERIENCE. AS YOU CAN SEE, THERE ARE MULTIPLE PROGRAMS OUTSIDE OF NIH. DARPA IS A BIG FUNDER. DARPA IS AN ACRONYM THAT I DON'T UNDERSTAND FULLY, BUT IT'S THE DEFENSE DEPARTMENT'S WAY OF FUNDING GRANTS, SMALL BUSINESSES INNOVATION RESEARCH GRANTS, ETC., WHERE THERE

ARE MULTIPLE AGENCIES WITH DIVERSE MISSIONS, AND MANY OF THEM FUND BIOMEDICAL RESEARCH. THERE IS NO ONE SIZE FITS ALL APPROACH AT THE FEDERAL LEVEL, BUT EACH OF THESE PROGRAMS HAS A SPECIFIC PURPOSE IN MIND WHEN THEY UNDERTAKE THEIR GRANT MAKING.

AND FINALLY, THE FEDERAL GOVERNMENT GENERALLY DOESN'T TREAT THE NONPROFIT COMPANIES DIFFERENTLY WITH RESPECT TO IP OR REVENUE SHARING, BUT OBVIOUSLY WE'LL HAVE OUR OWN DISCRETION OF HOW WE WOULD DO THAT IN THE STATE OF CALIFORNIA.

HERE'S THE AGENDA. YOU'VE JUST LISTENED TO MY INTRODUCTORY REMARKS. WE'RE PLEASED TO HAVE GREG MILMAN HERE TODAY FROM THE NIH TO DESCRIBE HIS EXPERIENCES AT NIH, AND YOU CAN READ THE REST OF THIS MENU HERE. IT'S A BUSY MORNING, SO WE'LL HAVE TO STAY ON TRACK. NO OFFENSE TO ANY OF THE OTHER PRESENTERS, BUT I THINK THAT GREG MILMAN'S, FIRST OF ALL, GREAT WILLINGNESS TO FLY OUT HERE TODAY FROM WASHINGTON WHERE HE LIVES AND WORKS TO MAKE THIS PRESENTATION, BUT ALSO REALLY A DEEP UNDERSTANDING OF THE FEDERAL PROGRAMS AND HOW THEY WORK, WE REALLY APPRECIATE GREG COMING.

AT THIS POINT, I'M JUST GOING TO INTRODUCE GREG. WE'D LIKE -- GREG WILL HAVE TO LEAVE AFTER HIS PRESENTATION, SO WE WANT TO -- GREG HAS SAID HE'S HAPPY TO HAVE COMMENTS AND QUESTIONS DURING HIS PRESENTATION

AND AFTER, BUT WE DON'T WANT TO SHORTCHANGE GREG'S PRESENTATION EVEN IF WE HAVE TO STEAL A LITTLE TIME FROM SOME OF OUR OTHER PRESENTERS. SO WITH THAT, I'LL ASK GREG TO COME FORWARD.

GREG IS A LONGTIME FRIEND, FACULTY MEMBER WITH ME AT BERKELEY IN THE EARLY '70S, AND THEN HE WAS A FACULTY MEMBER AT JOHNS HOPKINS FOR A NUMBER OF YEARS, WENT TO NIH TO THE AIDS PROGRAMS, AND DID THAT FOR MANY YEARS, AND HE'S RESPONSIBLE FOR SBIR PROGRAM AT NIH. SO GREG'S EXPERIENCE IS TERRIFIC, AND WE'RE VERY PLEASED TO HAVE GREG JOIN US TODAY. THANK YOU FOR COMING ALL THE WAY OUT TO DO THIS FOR US, GREG.

DR. REED: JOHN REED HERE. SORRY TO BE LATE.

DR. PENHOET: WE HAVE SEVERAL NEW ADDITIONS. MICHAEL GOLDBERG AND JEFF SHEEHY HAVE JOINED US IN SAN FRANCISCO.

DR. FONTANA: JEANNIE FONTANA TOO IN L.A.

CHAIRMAN PENHOET: WERE WE ABLE TO SEND COPIES OF GREG'S PRESENTATION TO ANY OF THE PEOPLE AT REMOTE SITES? SO YOU SHOULD HAVE A COPY OF GREG'S PRESENTATION, AND WE'RE STARTING WITH HIS FIRST SLIDE. SO, GREG, PLEASE. THANK YOU VERY MUCH.

DR. MILMAN: GOOD MORNING. THANK YOU FOR INVITING ME. I WILL TELL YOU WHEN I'M CHANGING SLIDES FOR THOSE PEOPLE WHO ARE AT REMOTE SITES OR THOSE WHO ARE

HERE. THIS LITTLE CARTOON CHARACTER THAT APPEARS ON MANY OF MY SLIDES IS DONE BY OUR ILLUSTRATOR, AND SHE CLAIMS THAT THE BALD-HEADED GUY WITH GLASSES HAS NO RESEMBLANCE TO ME, BUT I DON'T KNOW ABOUT THAT. BUT, ANYWAY, YOU CAN MAKE YOUR OWN DECISION ABOUT THAT. AND MY CONTACT INFORMATION AND E-MAIL IS THERE.

SO I HAVE TO GIVE A DISCLAIMER BEFORE I START. IT'S STANDARD TO SAY THAT MY OPINIONS DO NOT NECESSARILY REPRESENT THOSE OF NIH OR HHS. AND I WILL TRY TO KEEP THEM TO A MINIMUM AND GIVE YOU FACTS INSTEAD. BUT IF I DO HAVE OPINIONS AND YOU GET THEM, IT'S BASED ON MY EXPERIENCE. I'VE BEEN A FACULTY MEMBER FOR 30 YEARS AT A UNIVERSITY. STARTED A BIOTECHNOLOGY COMPANY, MANAGED BASIC AIDS RESEARCH FOR TEN YEARS, AND MANAGED THE NIAID SMALL BUSINESS PROGRAMS, A $100 MILLION A YEAR FOR ABOUT TEN YEARS. I ORGANIZED THE BIOENGINEERING CONSORTIUM, AND THAT WAS THE PRECURSOR TO THE BIOMEDICAL IMAGING AND BIOENGINEERING INSTITUTE. AND THE REASON THAT'S IMPORTANT IS THAT WAS REALLY THE FIRST STEP OF MOVING NIH FROM BASIC RESEARCH INTO APPLIED RESEARCH. SO THAT WAS A CHANGE IN HOW WE DO BUSINESS AND A CHANGE IN REVIEW COMMITTEES TO LOOK AT WHAT THE PROGRESS WOULD BE. AND I SIT IN ON THE BOARD OF BIOTECHNOLOGY, INDUSTRY ORGANIZATIONS, COUNCIL OF BIOTECHNOLOGY CENTERS.

SO WHAT I'M GOING TO DO THIS MORNING IS I'M

GOING TO TELL YOU, FIRST OF ALL, ABOUT OUR SMALL BUSINESS PROGRAMS AND WHY I THINK THEY ARE VERY VALUABLE. AND THEN I'M GOING TO TELL YOU HOW THEY ACTUALLY FUNCTION AND, FINALLY, HOW THEY MIGHT BE APPLIED TO WHAT CALIFORNIA HAS WITH CIRM.

SO FIRST OF ALL SOME BACKGROUND. WE HAVE TWO SMALL BUSINESS PROGRAMS. ONE'S CALLED SBIR, WHICH STANDS FOR SMALL BUSINESS INNOVATION RESEARCH, AND IT ACTUALLY STARTED IN 1982 AND WILL CONTINUE AT LEAST TO 2008 WHERE WE EXPECT IT TO BE REAUTHORIZED. THE KEY THING HERE IS TWO AND A HALF PERCENT OF ALL AGENCIES' RESEARCH DOLLARS THAT ARE EXTRAMURAL MUST GO TO SMALL BUSINESSES, SO THEY'RE A SET ASIDE. THEY ALWAYS GO TO SMALL BUSINESSES, AND THIS STARTED IN 1982.

NOW, THE ACADEMIC INSTITUTIONS SAID WE WANT TO BE INVOLVED IN SOME OF THESE SMALL BUSINESS PROJECTS. SO IN '92 THEY STARTED WHAT'S CALLED THE SMALL BUSINESS TECHNOLOGY TRANSFER RESEARCH PROGRAM. AND THIS IS A PROGRAM THAT REQUIRES THAT BUSINESSES COLLABORATE WITH UNIVERSITIES. THE IDEA BEING FOR BOTH OF THESE PROGRAMS WE WANT TRANSLATIONAL RESEARCH, THE STANDARD WORD IS BENCH TO BEDSIDE. WE WANT TO ACTUALLY MOVE RESEARCH FORWARD TO IMPROVE PUBLIC HEALTH.

ONE WAY OF DOING THIS IS TO GET EARLY COLLABORATION BETWEEN ACADEMIC SITES AND INDUSTRY. AND

SO THIS IS EXTENDED TO 2009. IT'S A SMALLER PERCENTAGE, .3 PERCENT OF EACH AGENCY'S EXTRAMURAL BUDGET.

AN INTERESTING THING ABOUT THESE IS ALTHOUGH THEY --

CHAIRMAN PENHOET: WHEN YOU SAY TWO AND A HALF PERCENT OF THE TOTAL NIH BUDGET BASICALLY?

DR. MILMAN: OF THE EXTRAMURAL BUDGET. IT'S ABOUT RIGHT NOW SOMEWHERE AROUND $800 MILLION A YEAR, OF WHICH, SINCE WE'RE THE SECOND LARGEST, WE MANAGE ABOUT A HUNDRED MILLION. THE INTERESTING THING ABOUT THESE, WHICH I THINK IS RELEVANT TO CIRM, IS THAT THEY'RE MANAGED BY THE SMALL BUSINESS ADMINISTRATION AS WELL AS THE FUNDING AGENCIES, OKAY, BECAUSE THE SMALL BUSINESS ADMINISTRATION WAS THE ONE THAT STARTED THESE PROGRAMS. AND THAT MEANS TWO THINGS. THEY HAVE DUAL PURPOSES. ONE OF THE PURPOSES IS THAT OF THE SBA, AND THAT'S ECONOMIC DEVELOPMENT. AND THEIR GOAL IS TO HAVE ECONOMIC DEVELOPMENT, NEW COMPANIES FORMED, AND NEW JOBS CREATED. AND THE GOAL OF THE AGENCIES, IN OUR CASE NIH, IS REALLY TO IMPROVE HEALTH. AND SOMETIMES THESE DON'T OVERLAP. THERE MAY BE DIFFERENCES OF OPINION ABOUT WHAT WE SHOULD DO.

I WOULD POINT OUT TO YOU THAT IT SEEMS TO ME VERY SIMILAR TO CIRM BECAUSE IN ONE CASE YOU WANT TO

UNDERSTAND BASIC UNDERSTANDING OF STEM CELLS, AND THE OTHER YOU WANT TO RETURN SOMETHING TO THE CITIZENS OF CALIFORNIA, WHICH COULD BE FUNDS OR IT COULD BE IMPROVEMENT IN HEALTH OR BOTH. SO YOU HAVE BOTH TYPES OF ISSUES HERE, WHICH MAY BE VERY SIMILAR TO THE ONES WE HAVE.

THIS IS JUST A LIST OF PARTICIPATING AGENCIES. AND YOU CAN SEE THAT ALL THE AGENCIES THAT DO RESEARCH AND GIVE FUNDS FOR RESEARCH HAVE TO HAVE SMALL BUSINESS PROGRAMS. THE NIH ONE COMES OUT OF HHS. SOME ONLY HAD SBIR PROGRAMS DOWN HERE ON THE BOTTOM, AND THE ONES ON THE TOP HAVE BOTH.

I APOLOGIZE TO THOSE PEOPLE OFF SITE. I FORGOT TO SAY I'M CHANGING SLIDES. IT'S HARD TO REMEMBER. SO I'M CHANGING THE SLIDE AGAIN.

SO THE SBIR PROGRAM HAS THREE PHASES. I THINK THIS IS INTERESTING FOR YOU TO CONSIDER. THE PHASE I IS THE PROOF OF FEASIBILITY. IT'S A SMALL AMOUNT OF DOLLARS. IT SAYS IN THE GUIDELINES THAT IT'S ABOUT A HUNDRED THOUSAND, BUT THE AVERAGE IS 160,000 A YEAR, AND IT'S ONE, SOMETIMES TWO YEARS, AND IT'S REALLY A PROOF OF CONCEPT. THE IDEA IS BEFORE YOU POUR A TON OF MONEY INTO SOMETHING, YOU WANT SOME EVIDENCE THAT IT REALLY IS GOING TO WORK. AND SO YOU HAVE A PHASE I FUNDING, WHICH HAS NOTHING TO DO WITH PHASE I CLINICAL TRIALS.

THOSE PEOPLE WHO GET PHASE I'S ARE THE ONLY ORGANIZATIONS THAT ARE ALLOWED TO APPLY FOR PHASE II. THAT'S WHERE THE MAJOR RESEARCH AND DEVELOPMENT TAKES PLACE. AND THE FUNDING IN PHASE II IS FOR TWO YEARS, SOMETIMES THREE, AND THE MEDIAN AWARD IS ABOUT 375,000 A YEAR. FOR BOTH OF THESE IT'S TOTAL COST, NOT DIRECT COST. SO IF THERE'S OVERHEAD INVOLVED, IT HAS TO COME OUT OF THESE DOLLARS.

NOW, FOR PROJECTS THAT ARE GOING TO LEAD TO PRODUCTS THAT REQUIRE FDA APPROVAL, WE CAN EXTEND THE PHASE II FUNDING. AND HERE'S WHERE THE DOLLARS GO UP EVEN MORE, AND THEY'RE CALLED COMPETING CONTINUATION PHASE II FOR THOSE PRODUCTS WILL HAVE TO GO TO THE FDA, AND THEY COMPETE WITH ALL THE OTHER APPLICATIONS. NOW WE'RE MAKING AWARDS UP TO A MILLION DOLLARS A YEAR FOR UP TO THREE YEARS, AND THESE CAN BE CONTINUED TO BE COMPETED. SO THE GOAL IS TO MOVE THE PROJECTS ALONG AS CLOSE AS YOU CAN TOWARDS CLINICAL TRIALS, NOT INTO CLINICAL TRIALS, AND THE GOAL IS TO ADD ENOUGH VALUE TO THE PROJECTS SO THAT ANGELS OR VC'S OR ANOTHER GROUP WILL TAKE OVER BECAUSE WE RECOGNIZE THAT THE AVERAGE COST OF PRODUCING A DRUG IS SOMETHING LIKE A BILLION DOLLARS ESTIMATED. AND THERE'S NO WAY THAT NIH CAN AFFORD THAT; BUT IF WE ADD ENOUGH VALUE TO THE PROJECT, SOMEBODY ELSE MAY.

AND WHO'S THE SOMEONE ELSE? WELL, THAT'S THE PHASE III. IT'S THE REMAINING STEPS TO COMMERCIALIZATION WHERE YOU HAVE TO ROUND UP THE BIG DOLLARS FROM SOME OTHER SOURCE. AND IT'S NOT FUNDED BY THE GOVERNMENT. IT'S FUNDED BY OTHER SOURCES, SAY, ANGELS OR VC'S. SO THAT'S HOW THE WHOLE PROGRAM IS SET UP TO FUNCTION.

SO WHAT I'M GOING TO TELL YOU NOW IS SOME MEASURES, SURROGATE MEASURES, OF PROGRAM VALUE. IT'S VERY HARD TO MEASURE THE SUCCESS OF THE SMALL BUSINESS PROGRAMS BECAUSE IT'S DIFFICULT TO FOLLOW A PROJECT ALL THE WAY FROM INCEPTION IN PHASE I ALL THE WAY TO DOWN THE ROAD. AND THE COMPANIES OFTEN ARE MERGED OR ACQUIRED BY SOMEBODY ELSE ALONG THE WAY, AND IT'S VERY DIFFICULT, ALTHOUGH THE NATIONAL ACADEMY HAS TRIED TO DO IT, AND THERE'S SOME EVIDENCE THAT THEY'RE SUCCESSFUL AT MEASURING SOME OF IT, BUT I'M GOING TO GIVE YOU WHAT I THINK ARE THREE SURROGATES THAT MIGHT INDICATE THE SUCCESS OF THE PROGRAM.

THE FIRST IS LEVERAGE. HOW MUCH MONEY IS COMING INTO COMPANIES THAT GET SBIR COMPARED TO THE MONEY THAT THE GOVERNMENT IS PUTTING IN? THAT'S THE FIRST ONE. IF YOU CAN GET LOTS OF EXTRA MONEY IN, IT'S INDICATING THAT THE GOVERNMENT IS PUTTING IN A SMALL AMOUNT OF MONEY AND THAT'S MATCHED A LOT BY SOMEONE ELSE. SO THAT'S GOOD. YOU'RE MOVING YOUR RESEARCH ALONG WITHOUT PAYING FOR IT.

THE SECOND IS PATENTS. HOW ARE THE PATENTS THAT THE SMALL BUSINESS COMPANIES GET COMPARED TO THE PATENTS UNIVERSITIES GET, REMEMBERING THAT THE UNIVERSITIES ARE GETTING 97 PERCENT OF THE FUNDS AND THE SMALL BUSINESSES ARE ONLY GETTING 3 PERCENT OF THE FUNDS. HOW DO THEY COMPARE?

THIRD IS WHAT'S THE FINANCIAL HEALTH OF THESE COMPANIES? AND THAT'S A HARD ONE TO MEASURE. AND SO WHAT I'VE TAKEN HERE AS A SURROGATE IS WHAT ABOUT THE COMPANIES THAT HAVE JOINED THE BIOTECHNOLOGY INDUSTRY ORGANIZATION? NOW, THAT ORGANIZATION CHARGES FIVE, $10,000 OR MORE TO BE A MEMBER FOR COMPANIES. IN ORDER FOR A COMPANY TO JOIN, THEY MUST BE PRETTY HEALTHY. SO LET'S LOOK AT THE ONES THAT JOIN AND ASK WHAT PERCENTAGE OF THEM ACTUALLY GOT SMALL BUSINESS GRANTS. THAT GIVES YOU AN IDEA THAT THEY ACTUALLY HAVE BEEN SUCCESSFUL. OKAY.

SO HERE'S SOME NUMBERS WHICH I THINK ARE VERY INTERESTING. THEY COME FROM THIS COMPANY CALLED INKNOWVATION.COM. ANN ESKESEN IS THE ONE WHO STARTED IT, AND SHE ACTUALLY IS THE ONE WHO ORIGINATED THE IDEA OF THE SMALL BUSINESS PROGRAM AND CONVINCED CONGRESS TO FUND IT IN 1982. AND SHE FOLLOWS THESE SMALL BUSINESSES OF ALL DIFFERENT KINDS, AND YOU CAN GET MORE DATA FROM HER ORGANIZATIONS, BUT SHE'S MANAGED TO PUT THIS TOGETHER FOR

ME SO I COULD PRESENT IT TO YOU.

WHAT DO WE HAVE HERE? THIS IS THE SOURCE ON THE LEFT-HAND SIDE OF THE SBIR FUNDING. AND WHEN I SAY ALL SOURCES, THAT'S ALL THE AGENCIES, INCLUDING DOD, DEPARTMENT OF COMMERCE, AND WHATEVER. AND YOU CAN SEE THAT FROM 2002 TO PRESENT, THERE WERE ABOUT 7,700 OF THESE COMPANIES. HOW MANY ATTRACTED VC FUNDS? SHE ACTUALLY HAS A MEASUREMENT OF ALL OF THESE, AND IT TURNS OUT ABOUT 11 PERCENT ACTUALLY GOT MONEY FROM VENTURE CAPITALISTS.

IF YOU LOOK AT NIH, WHICH IS A MEASURE OF FUNDING IN THE BIOLOGICAL AREA, THE HEALTH AREA, YOU SEE THERE ARE 3,000 COMPANIES THAT GOT FUNDED, OF WHICH ABOUT 17 PERCENT GOT VC FUNDING. SO THIS INDICATES NOW THAT YOU'RE GETTING LEVERAGE FROM THE VC'S. SO WHAT I DID IS I BROKE IT OUT ALSO BY CALIFORNIA BECAUSE THERE ARE MORE VC'S HERE AND MORE BIOTECH COMPANIES HERE. HOW WELL DID THEY DO?

IF YOU LOOK AT NIH, THERE'S 601 COMPANIES IN CALIFORNIA THAT RECEIVED SMALL BUSINESS FUNDS, AND ABOUT 28 PERCENT OF THEM MANAGED TO GET VC FUNDING. THAT INDICATES THAT THE LEVERAGE IS THERE, THAT THE GOVERNMENT IS PUTTING IN MONEY, BUT ALSO VC'S ARE PUTTING IN MONEY. SO HOW MUCH MONEY ARE THEY PUTTING IN? WELL, I THINK THIS IS A REAL TELLING POINT. HERE IS ALL 50 STATES

AGAIN. LOOK AT THE NIH DOLLARS, AND YOU CAN SEE THAT OVER THAT PERIOD OF TIME, NIH PUT IN ABOUT $1.6 BILLION IN SMALL BUSINESSES. AND OF THOSE BUSINESSES THAT GOT VC MONEY, THEY GOT $13.7 BILLION OR ABOUT NINE TIMES AS MUCH MONEY CAME FROM THE VC'S AS COME FROM THE GOVERNMENT. SO YOU'RE GETTING NINE TIMES AS MUCH FOR YOUR MONEY AS YOU WOULD GET IF YOU JUST PUT IT IN AND NOBODY ELSE LEVERAGED IT WITH YOU.

WHAT ABOUT CALIFORNIA? WELL, LOOK AT THIS. IT'S EVEN MORE. IT'S ALMOST 18 TIMES AS MUCH MONEY COMING FROM VC'S AS IS COMING FROM THE FEDERAL GOVERNMENT. AND THAT'S LEADING TO THE ECONOMIC DEVELOPMENT AND PRODUCTION OF WHATEVER PRODUCTS THAT YOU MIGHT HAVE. SO THAT IS A GOOD REASON TO INDICATE THAT THE SMALL BUSINESS PROGRAM IS DOING A GOOD JOB.

CHAIRMAN PENHOET: MAYBE SAYS VC'S OUGHT TO MOVE SOMEWHERE ELSE. THERE'S TOO MANY OF THEM.

DR. PIZZO: NO. IT SUGGESTS THAT YOU SHOULD PUT MORE SBIR FUNDS INTO CALIFORNIA BECAUSE IT HAS A HIGHER RETURN. JUST FORGET ABOUT THE REST OF THE COUNTRY.

DR. MILMAN: HERE'S ANOTHER INTERESTING FACT. THESE ARE THE COMPANIES SINCE 1982 OR 83 THAT WERE FUNDED WITH SMALL BUSINESS FUNDS. YOU CAN SEE THE NUMBER HAS GROWN. THIS IS ALL SMALL BUSINESS FUNDS, NOT JUST HEALTH FUNDS. TO A LARGE NUMBER OF COMPANIES ON THE LEFT-HAND

SIDE, THE NUMBER OF PATENTS -- DON'T HAVE THE PATENTS. HERE'S THE PATENTS FROM THE SMALL BUSINESS COMPANIES. THEY'RE ON THE RIGHT-HAND SIDE INDICATED BY THE RED SQUARES. AND THAT'S COMING OUT OF THESE COMPANIES. AND HERE'S THE PATENTS COMING OUT OF UNIVERSITIES. AND YOU CAN SEE THAT NOT ONLY DO THE SMALL BUSINESSES HAVE THE SAME NUMBER OF PATENTS AS UNIVERSITIES UP UNTIL ABOUT 1998-99, BUT THEY'VE EXCEEDED IT SINCE THEN.

DR. PIZZO: ISN'T THAT A SELECTIVE BIAS BECAUSE YOU ARE MOVING BASIC RESEARCH THAT YOU THINK HAS --

DR. MILMAN: OF COURSE. I ONLY ARGUE THAT THIS IS A SURROGATE. I CAN'T TELL EXACTLY WHERE THOSE PATENTS ARE COMING FROM. THERE'S CERTAINLY NO INDICATION THAT THEY'RE COMING FROM THE SMALL BUSINESS FUNDS. WE DON'T KNOW THAT. BUT IT IS INTERESTING TO SEE THAT AT LEAST THEY'RE KEEPING UP WITH THE UNIVERSITIES. THERE'S A LARGE NUMBER OF PATENTS. REMEMBER, I STARTED BY SAYING THESE ARE INACCURATE SURROGATES, BUT THE BEST THAT I COULD COME UP WITH.

CHAIRMAN PENHOET: THEY'RE NOT NORMALIZED. THESE ARE GROSS NUMBER OF PATENTS PER DOLLAR.

DR. MILMAN: PER DOLLAR THE SMALL BUSINESSES WHO GET VC FUNDING ARE DOING MUCH BETTER. REMEMBER, THEY'RE ONLY GETTING 3 PERCENT OF OUR DOLLARS. SO THE ARGUMENT THAT I'M MAKING IS THAT THEY'RE DOING PRETTY WELL

CONSIDERING. UNIVERSITIES ARE DOING BASIC RESEARCH. OFTENTIMES THAT DOESN'T LEAD TO PATENTS AT ALL. BUT I WOULD STRONGLY ARGUE THAT WITHOUT PATENTS, YOU CAN'T DEVELOP PRODUCTS. WITHOUT PATENTS YOU WON'T GET VC'S INVOLVED IN ACTUALLY FUNDING THE COMPANIES OR FUNDING THE FOLLOW-ON RESEARCH, SO IT'S REALLY ESSENTIAL THAT YOU HAVE THE PATENTS THAT ARE HERE.

AND THE LAST INFORMATION, THE LAST SURROGATE THAT I HAVE IS ON THE BIO MEMBERS. AND I DID THIS LAST YEAR ABOUT THIS TIME. THERE WERE 1150 BIO MEMBERS OR SO, AND ABOUT 16.3 PERCENT OF MEMBERS RECEIVED SBIR FUNDING. THAT'S A PRETTY HIGH PERCENTAGE, PARTICULARLY WHEN YOU RECOGNIZE THAT OVER 50 PERCENT OF THOSE MEMBERS ARE INELIGIBLE BECAUSE THEY'RE EITHER FOREIGN COMPANIES OR THEY'RE TOO BIG TO RECEIVE SBIR FUNDS. SO WHAT WE ACTUALLY HAVE --

CHAIRMAN PENHOET: WHAT IS THE CUTOFF ON SIZE FOR SBIR?

DR. MILMAN: CUTOFF IN SIZE IS 500 EMPLOYEES, BUT THAT'S REALLY NOT THE MAJOR CUTOFF. THE MAJOR CUTOFF IS THE COMPANY MUST BE OWNED OVER HALF BY U.S. CITIZENS. AND THAT MEANS THE VC'S, COMPANIES, WHEN VC'S INVEST MONEY IN COMPANIES, THEY USUALLY TAKE OVER 50 PERCENT. THEREFORE, THE COMPANY BECOMES INELIGIBLE.

MR. GOLDBERG: THAT'S A DIFFERENT TEST.

DR. MILMAN: THAT'S ANOTHER TEST. BASICALLY THAT'S THE ONE THAT USUALLY ELIMINATES THE COMPANIES FROM THIS. WHEN I SAY VC'S PUT MONEY INTO IT, IT'S AFTER THE NIH HAS PUT MONEY INTO IT. IT'S NOT BEFORE.

SO PROBABLY OVER HALF OF THE COMPANIES THAT WERE ELIGIBLE FOR FUNDING ACTUALLY RECEIVED THE MONEY.

HERE'S THE TAKE-HOME MESSAGE FOR THIS PART OF THE TALK. I THINK THE FEDERAL SBIR-STTR PROGRAMS SEEM TO BE AN EFFECTIVE WAY TO LEVERAGE GOVERNMENT INVESTMENTS. THEY PROMOTE TRANSLATIONAL RESEARCH AND ECONOMIC DEVELOPMENT.

THE SMALL BUSINESS FUNDING OF COMPANIES IN CALIFORNIA WAS MATCHED ABOUT SIXFOLD BY VENTURE CAPITAL FUNDING. IF YOU TAKE THE PERCENTAGE OF THOSE THAT GOT VC'S AND HOW MUCH THEY GOT, YOU'RE GETTING SIX TIMES YOUR DOLLAR'S WORTH OF MONEY COMING IN, WHICH I THINK IS PRETTY IMPRESSIVE.

AND THE SBIR COMPANIES TODAY ARE AWARDED MORE PATENTS THAN UNIVERSITIES. THAT INDICATES THE PATENTS LEADING TO PRODUCTS IS COMING OUT OF THESE COMPANIES. AND A HIGHER PERCENTAGE OF THE BIOTECHNOLOGY COMPANIES WHO CAN AFFORD TO BELONG TO BIO WERE AWARDED THESE GRANTS, SO IT INDICATES THEY HAVE BEEN SUCCESSFUL. MANY OF THESE START OUT, AND I SHOULD SAY MANY OF THEM START OUT WITH THEIR FIRST FUNDING COMING FROM SBIR AND STTR.

THEY OFTEN START OUT AS ACADEMIC PEOPLE WHO SET UP COMPANIES, GET FUNDING FROM THE FEDERAL GOVERNMENT TO START THESE COMPANIES WITHOUT DILUTING ANY CAPITAL, AND THEN MOVE FORWARD.

WHAT YOU'RE INTERESTED IN IS OWNERSHIP OF THE INTELLECTUAL PROPERTY, RIGHT, PATENTS. AND WHAT I'M GOING TO TELL YOU IS THAT THE BAYH-DOLE ACT SAYS THAT THE PATENTS, AS ED HAD ALREADY SAID, BELONG TO THE COMPANIES. AND WE DO NOT HAVE ANY LICENSING. AND THE THEORY BEHIND IT, IN MY OPINION, IS THAT WE GAIN BY IMPROVING PUBLIC HEALTH AND WE GAIN BY CREATING JOBS AND CREATING TAX BASE WHERE THE GOVERNMENT MAKES THE MONEY BACK. IF WE WERE TO TAKE LICENSING FUNDS FROM THESE COMPANIES, IF WE WERE TO ASK THEM FOR IT, IT WOULD BE MUCH HARDER FOR THEM TO RAISE THE VC FUNDING, WHICH YOU CAN SEE IS MANY TIMES WHAT WE'RE PUTTING INTO THEM. THEREFORE, THE CRITICAL THING HERE IS WE WANT THE LEVERAGE MORE THAN ANYTHING ELSE, AND WE WANT THE JOBS AND WE WANT THE ECONOMIC DEVELOPMENT.

NOW, YOU CAN'T JUST HAVE A COMPANY COME IN AND DECIDE TO DO WHATEVER THEY WANT. THEY ACTUALLY HAVE TO REPORT TO THE GOVERNMENT ANY INVENTION THEY MAKE, AND THEY MUST PURSUE THE PATENT APPLICATION. IF THEY DON'T PURSUE IT, THE GOVERNMENT CAN TAKE IT OVER AND PURSUE IT ITSELF. EACH GRANTING AGENCY HAS THE RIGHT TO MARCH IN

AND TAKE OVER PURSUING THE PATENT IF THE GRANTEE COMPANY DOESN'T DO IT. SO THAT'S A GOOD INCENTIVE FOR COMPANIES TO PAY MONEY. IT'S VERY EXPENSIVE TO ACTUALLY GET PATENTS. SO THIS PUSHES THEM ACTUALLY INTO APPLYING FOR PATENTS TO MAKE SURE THAT THEY ACTUALLY HAVE THOSE PATENTS.

MR. GOLDBERG: AND DOES THIS UMBRELLA POLICY EXTEND TO ALL AGENCIES?

DR. MILMAN: ALL AGENCIES.

MR. GOLDBERG: SO THERE IS NO FLEXIBILITY ON THE PART OF THE VARIOUS --

DR. MILMAN: EVERY AGENCY IS THE SAME. SO IF THE COMPANY DOESN'T WANT TO PURSUE A PATENT AND THE GOVERNMENT DOESN'T WANT TO PURSUE A PATENT AND THE INVENTOR STILL THINKS THAT THIS IS AN IMPORTANT THING TO DO, WHICH SOMETIMES HAPPENS, THEN THE INVENTOR CAN GO AHEAD AND PURSUE THE PATENT.

DR. STEWARD: AS LONG AS YOU STOPPED FOR A SECOND, CAN YOU SAY SOMETHING ABOUT THE TIMING OF THAT, OF THE MARCH-IN RIGHTS? HOW LONG A WINDOW DOES THE COMPANY HAVE BEFORE THAT IS EXERCISED?

DR. MILMAN: I THINK THEY HAVE SOMETHING LIKE A YEAR. THEY HAVE A PERIOD OF TIME. IT'S NOT CLEAR. OBVIOUSLY THEY DON'T HAVE A LONG TIME BECAUSE YOU ACTUALLY HAVE TO FILE A PATENT IN A PERIOD OF TIME. I'M

NOT SURE ABOUT IT EXACTLY. I CAN SAY PROBABLY, AT LEAST FOR NIH, THAT THEY'VE NEVER EXERCISED MARCH-IN RIGHTS. THIS IS A THREAT. IT'S NOT SOMETHING THAT YOU ACTUALLY DO. AND THE REASON IT'S THERE, YOU CONSIDER IT, IS TO MAKE SURE THINGS GET PATENTED BECAUSE YOU HAVE THE THREAT TO DO IT YOURSELF. SO IT DOESN'T REALLY MATTER AS LONG AS THE COMPANIES GO AHEAD AND DO IT.

HERE'S ANOTHER THING THAT WE NEVER DO. THE GRANTING AGENCY HAS THE RIGHT TO A ROYALTY FREE LICENSE TO PRACTICE THE INVENTION FOR ITS OWN USE. SO THEORETICALLY THE HAMMER IS THERE THAT SAYS, YOU KNOW, IF YOU DON'T DO THIS APPROPRIATELY, WE CAN TAKE IT OVER AND HAVE SOMEBODY ELSE DO IT FOR YOU, AND NIH HAS NEVER DONE THIS, ALTHOUGH THERE HAVE BEEN THREATS TO DO IT DURING THE ANTHRAX ERA AND CIPRO, WHICH CONVINCED THE COMPANIES TO LOWER THE PRICE. SO THERE IS LEVERAGE THERE.

AND HERE, I THINK, IS AN IMPORTANT ISSUE HERE, WHICH I BRING TO YOUR ATTENTION IS THE PRODUCTS THAT COME OUT OF THESE GRANTS HAVE TO BE PRODUCED IN THE U.S. SO THE ANALOGY IS IF YOU DID IT IN CALIFORNIA, THE PRODUCTS WOULD HAVE TO BE PRODUCED IN CALIFORNIA SO THAT IN CALIFORNIA YOU HAVE ECONOMIC DEVELOPMENT.

CHAIRMAN PENHOET: GREG, OWN USE MEANS WHAT IN THIS CONTEXT?

DR. MILMAN: WELL, WHAT IT MEANS IS IT MAY BE

USED BY DOD, IF THEY CAN'T GET THE COMPANY TO MAKE SOME PRODUCT THAT THEY ACTUALLY NEED. THE OWN USE MIGHT BE, AS I SAID, THE GOVERNMENT SAYS THAT CIPRO COSTS WAY TOO MUCH, AND WE NEED TO MANUFACTURE IT WITHOUT GOING THROUGH THE RIGHT COMPANY BECAUSE IT'S A PUBLIC HEALTH HAZARD NOT TO HAVE IT. IT'S A DIFFICULT THING. AND AS FAR AS I KNOW, IT'S NEVER BEEN USED, BUT IT'S REALLY THE HAMMER TO GET STUFF DONE.

DR. PIZZO: GREG, I THINK THIS IS SELF-EVIDENT, BUT FOR THE LAST STATEMENT WHERE IT'S PRODUCED IN THE U.S., PRESUMABLY THAT MEANS THAT IT CAN BE DISTRIBUTED ANYWHERE.

DR. MILMAN: ANYWHERE, YES. IT'S AN ECONOMIC DEVELOPMENT ISSUE.

DR. PIZZO: SO THE CALIFORNIA ANALOGY IS BEING PRODUCED IN CALIFORNIA, SHARED --

DR. MILMAN: EVERYWHERE. BUT THE GOAL IS THAT THE MONEY THAT WOULD COME FROM IT WOULD TAKE PLACE IN CALIFORNIA, JUST THE WAY IT TAKES PLACE HERE AND THE JOB WOULD TAKE PLACE HERE. THE ISSUE, I THINK, IT'S MY OPINION, THAT BY DOING THE WAY THAT GOVERNMENT DOES IT, WE'RE NOT GETTING ACTUALLY LICENSING, BUT WE'RE ENSURING ECONOMIC DEVELOPMENT TAKES PLACE IN THIS COUNTRY.

SO NOW I'M GOING TO TELL YOU ABOUT THE SMALL BUSINESS PROGRAM AND REQUIREMENTS AND HOW IT FUNCTIONS,

JUST SO YOU CAN LOOK AT IT AND SEE HOW YOU MIGHT WANT TO DO IT IF YOU WERE GOING TO DO THAT SAME TYPE OF PROGRAM IN CALIFORNIA.

FIRST OF ALL, IT ONLY GOES TO BUSINESSES. THEY HAVE TO BE FOR PROFIT. THE PRINCIPAL PLACE OF THE BUSINESS HAS TO BE IN THE U.S. YOU WILL NOTICE I'VE HIGHLIGHTED IN RED HERE THOSE AREAS THAT YOU MIGHT WANT TO CHANGE TO CALIFORNIA. THE FUNDED RESEARCH MUST BE CONDUCTED ENTIRELY IN THE U.S. YOU CAN'T TAKE THIS MONEY AND TAKE IT ABROAD. WE WANT THE MONEY TO BE USED HERE. A REASONABLE PORTION OF THE RESEARCH MUST BE CONDUCTED BY THE COMPANY IN COMPANY-CONTROLLED FACILITIES. THIS IS TO PREVENT VIRTUAL COMPANIES. THE GOAL OF THE SBA IS TO DEVELOP REAL COMPANIES THAT MAKE REAL PRODUCTS AND DO IT WITH REAL RESOURCES AND HIRE REAL PEOPLE AS OPPOSED TO HAVING A BUNCH OF ACADEMIC LABS DOING THE WORK.

IT HAS TO BE SMALL, ALTHOUGH FOR BIOTECH COMPANIES 500 OR FEWER IS NOT THAT SMALL. AND HERE'S AN INTERESTING THING THERE'S A LOT OF DEBATE ABOUT. AT THE PRESENT TIME THE COMPANIES MUST BE OWNED BY INDIVIDUAL U.S. CITIZENS AND NOT VENTURE CAPITAL ORGANIZATIONS. THE REASON THAT'S THERE IS FROM THE SBA BECAUSE THEIR GOAL IS TO PROMOTE COMPANIES AND TO PUT MONEY IN THE ONES THAT ARE GOING TO GROW THE MOST. AND THE THEORY BEING THAT AFTER THEY GET THE VC MONEY, SOMEBODY ELSE IS GOING TO

FORCE THEM TO GROW. BEFORE, WHEN THEY'RE GETTING THIS MONEY, THE GOVERNMENT IS PUTTING IT IN, WE WANT TO ADD ENOUGH VALUE SO VC'S WILL PUT IN THAT TEN TIMES AS MUCH AMOUNT. WHEREAS, THE AGENCIES, AND I MUST ADMIT BEING AN AGENCY PERSON, FEEL THAT WE'D ACTUALLY LIKE TO FUND THOSE COMPANIES THAT HAVE THE BEST CHANCE OF SUCCESSFULLY PRODUCING A PRODUCT. AND SOMETIMES THOSE ARE AND OFTENTIMES THOSE ARE THE VC COMPANIES BECAUSE THEY'VE GOT THE MANAGEMENT AND THE STRUCTURE IN TO ACTUALLY TAKE IT ALL THE WAY TO COMMERCIALIZATION. SO YOU'VE GOT THAT ISSUE HERE BETWEEN THE TWO THAT I WANTED TO POINT OUT.

DR. STEWARD: CAN I ASK A QUESTION THERE? GOING BACK TO THAT SLIDE, WHAT DOES PRINCIPAL PLACE OF BUSINESS MEAN? IS THERE A PROPORTION THAT YOU LOOK FOR?

DR. MILMAN: IT MEANS THAT WHERE THE WORK IS DONE HAS TO BE IN THE U.S. AND IT CAN'T BE A SUBSIDIARY OF A FOREIGN COUNTRY, A FOREIGN COMPANY.

DR. STEWARD: I'M JUST TRYING TO THINK ABOUT THE CALIFORNIA SITUATION.

DR. MILMAN: SO THE FUNDS ARE FOR INNOVATIVE RESEARCH, REALLY NOT DEVELOPMENT. THAT'S NEW TECHNOLOGIES, IMPROVING EXISTING TECHNOLOGIES, NEW APPLICATIONS. WHAT I TELL PEOPLE IS RESEARCH IS JUST THE COLLECTION AND ANALYSIS OF DATA. IT'S TO VALIDATE A PRODUCT, BUT NOT NECESSARILY TO BUILD A BETTER WIDGET,

AND IT'S REALLY NOT FOR DEVELOPMENT, ALTHOUGH DEVELOPMENT MAY BE AN IMPORTANT PART OF THE PROJECT. THE FUNDING IS REALLY FOR THE RESEARCH PART.

HERE'S THE INFORMATION ON THE TWO DIFFERENT PROGRAMS JUST SO YOU GET SOME IDEA. WE ALREADY SAID THAT TWO AND A HALF PERCENT GOES TO SBIR; .3 PERCENT GOES TO STTR. THE AWARD GUIDELINES ARE NOT EXACTLY -- WELL, THEY'RE THERE. A 100,000 FOR SIX MONTHS OR 12 MONTHS FOR PHASE I, BUT PEOPLE USUALLY GET MORE THAN THIS. PHASE II'S ARE 750,000 FOR TWO YEARS, BUT THEY GET MORE THAN THAT TOO USUALLY, SO THOSE ARE SORT OF THE NORMAL AMOUNTS.

THE DIFFERENCES IN THE STTR IS YOU HAVE TO HAVE A RESEARCH INSTITUTION AS A PARTNER. AND BECAUSE YOU HAVE A RESEARCH INSTITUTION AS A PARTNER, YOU CAN OUTSOURCE MORE OF THE WORK, BUT NOT ALL OF THE WORK. YOU WILL NOTICE HERE THAT IN THE OUTSOURCING, THE MAXIMUM AMOUNT YOU CAN OUTSOURCE AS A COMPANY IS 60 PERCENT FOR THE STTR AND A THIRD FOR THE SBIR. COMPANIES HAVE TO DO WORK. IT CAN'T BE VIRTUAL. THAT'S THE CRITICAL THING HERE. AND THERE ARE MINIMUM COMPANY EFFORTS AND MINIMUM RESEARCH INSTITUTION EFFORTS.

THE OTHER KEY THING HERE --

CHAIRMAN PENHOET: THE RESEARCH INSTITUTION HERE IS DEFINED AS THE NOT-FOR-PROFIT ACADEMIC.

DR. MILMAN: ACADEMIC. NOT FOR PROFIT. EXACTLY.

THE CRITICAL THING HERE IS THAT IN AN SBIR, THE PRINCIPAL INVESTIGATOR MUST BE EMPLOYED BY THE COMPANY OVER HALF-TIME. MUST BE. THAT'S JUST PROVING THAT IT'S A REAL COMPANY AND THEY HAVE AN INVESTIGATOR.

FOR THE STTR, THEY'RE OFTEN ACADEMIC INVESTIGATORS WHO ARE THE PRINCIPAL INVESTIGATORS. THEY DON'T EVEN NEED TO GET A SALARY FROM THE COMPANY. THEY NEED TO BE AFFILIATED WITH A COMPANY. SO THAT'S THE MAJOR REASON FOR STTR'S.

IN FACT, I'LL SHOW YOU IN A MINUTE. WE ALSO HAVE WHAT'S CALLED FAST TRACK. REMEMBER I TOLD YOU THERE'S PHASE I AND PHASE II. AND HERE'S HOW THE PHASE I WORKS. YOU SUBMIT YOUR APPLICATION, YOU WAIT SEVEN TO NINE MONTHS FOR THE GOVERNMENT FOR REVIEW, AND YOU GET AN AWARD WHICH IS SIX MONTHS TO A YEAR. AND AFTER IT'S OVER, YOU HAVE TO PREPARE A WHOLE OTHER APPLICATION AND SUBMIT IT FOR THE PHASE II. SO THERE'S THIS DELAY TIME BECAUSE YOU CAN'T DO ANYTHING WHILE YOU'RE WAITING FOR THE PHASE II TO BE CONSIDERED. SO SOMEWHERE SIX TO NINE MONTHS WHILE YOU'RE WAITING FOR THE REVIEW TO TAKE PLACE, AND THEN YOU CAN GO AHEAD AND GET AN AWARD. SO THERE'S THIS GAP BETWEEN PHASE I AND PHASE II.

WE FOUND THAT REAL COMPANIES ACTUALLY HAVE THEIR

OWN FUNDS AND THEY CARRY ON DURING THIS GAP. NORMALLY IN SOME OF THE STATES, LIKE NEW YORK AND PENNSYLVANIA, THE STATE WILL ACTUALLY DO AN AWARD TO A COMPANY THAT GETS A PHASE I SO THAT THEY HAVE THIS FUNDING IN THE GAP BETWEEN PHASE I AND PHASE II. FAST TRACK IS JUST A PROCESS WHERE YOU SUBMIT BOTH PHASE I AND PHASE II APPLICATIONS AT THE SAME TIME. THEY GET REVIEWED, THEY HAVE MILESTONES, YOU GET AN AWARD, YOU DO A PROGRESS REPORT. THE PROGRAM COMES IN AND MAKES A DECISION OF WHETHER YOU MET YOUR MILESTONES; AND IF IT DOES, THEN YOU CAN GET AN AWARD, AND THAT'S ABOUT SEVEN MONTHS EARLIER, SO WE NARROWED THE GAP.

AND THOSE ARE THE TWO DIFFERENT PROGRAMS WE HAVE.

CHAIRMAN PENHOET: HOW DO YOU QUALIFY FOR FAST TRACK?

DR. MILMAN: WELL, YOU NEED TO HAVE, IN MY OPINION, YOU NEED TO HAVE A PROJECT WHERE IT'S VERY CLEAR WHAT THE MILESTONES ARE BECAUSE THE CRITICAL THING ABOUT THE FAST TRACK IS THE REVIEWERS WANT TO MAKE SURE THAT YOU'VE ACTUALLY MET YOUR MILESTONES. IF THEY DON'T THINK THAT YOU -- THEY WANT TO SEE THE RESULTS BEFORE THEY GIVE YOU THE MONEY FOR THE PHASE II, THEN THEY WANT YOU TO COME BACK. I SHOULD REPHRASE THAT AND SEE IF I CAN DO THAT AGAIN.

REMEMBER THE PHASE I IS PROOF OF CONCEPT. AND SOMETIMES REVIEWERS SAY I WANT TO SEE THE PROOF OF CONCEPT. I DON'T WANT THOSE PROGRAM PEOPLE TO MAKE THE DECISION. AND SO IF THEY NEED TO COME BACK AND SEE IT, THEY WON'T GIVE YOU A FAST TRACK. BUT IF IT'S ABSOLUTELY CLEAR, FOR EXAMPLE, IN DRUG DEVELOPMENT, YOU'VE ALREADY GOT THE COMPOUND DECIDED AND YOU'RE GOING TO DO BIOAVAILABILITY AND YOU ARE GOING TO DO TOXICOLOGY AND YOU KNOW THAT IF IT DOESN'T WORK, YOU'RE GOING TO STOP IT, THEN IT'S PRETTY CLEAR THERE'S NOTHING MUCH YOU HAVE TO LOOK AT. IF YOU HAVE AN ASSAY AND YOU'RE LOOKING FOR THE LEAD COMPOUND, THEN THE REVIEWERS WOULD LIKE TO SEE DID YOU REALLY FIND ONE THAT THEY THINK IS USEFUL BEFORE THEY FUND FURTHER. MAKE SENSE?

HERE'S THE ADVANTAGES OF THE SBIR. YOU DON'T NEED AN INSTITUTION PARTNER, WHICH MEANS THAT LAWYERS ARE LESS INVOLVED, WHICH IS A BIG THING FOR COST, AND THE COMPANY CONTROLS ALL THE FUNDS. AND MOST OF THESE COMPANIES HAVE -- WELL, THE MAXIMUM OVERHEAD THEY'RE ALLOWED TO ASK FOR IN PHASE I IS 25 PERCENT, SO IT DOES LOWER THE OVERHEAD COMPARED TO UNIVERSITIES THAT CAN BE ANYWHERE FROM 50 TO A HUNDRED PLUS PERCENT. AND IT'S MORE FLEXIBLE ON THE PERCENT EFFORTS THAN THE STTR.

AND THE ADVANTAGE IN THE STTR'S IS REALLY THAT ACADEMIC INVESTIGATOR WHO'S GOING TO BE THE PRINCIPAL

INVESTIGATOR. THAT'S THE MAJOR ADVANTAGE. SOMEBODY FROM THE UNIVERSITY IS GOING TO ACTUALLY RUN THE PROGRAM WITH THE COMPANY, COLLABORATION.

AND THAT MAY BE IMPORTANT TO THE PERSON RUNNING THE PROGRAM FOR PROMOTION, AND IT MAY BE EASIER TO AVOID CONFLICT OF INTEREST, SO THAT'S WHY AN INVESTIGATOR WANTS TO GET INVOLVED. AND IT MEANS THAT THE COMPANY MIGHT HAVE BETTER ACCESS TO ACADEMIC FACILITIES, INTELLECTUAL PROPERTY, IRB'S, ANIMAL WELFARE COMMITTEES, ALL THOSE THINGS THAT UNIVERSITIES ALREADY HAVE IN THEIR INDIRECT COST THAT THEN THE COMPANIES CAN USE AND A HIGHER PERCENT OF THE SUBCONTRACT IS POSSIBLE. SO THOSE ARE THE TWO PROGRAMS.

AND ACTUALLY IT TURNS OUT THAT WE GET FEWER APPLICATIONS FOR STTR, SO THE PROBABILITY OF FUNDING IS A LITTLE BETTER, BUT THAT COULD BE A THING THAT WILL CHANGE. THEY DO REQUIRE EXTRA EFFORT. OBVIOUSLY YOU HAVE PARTNERS HERE, SO BOTH THE COMPANY AND THE RESEARCH INSTITUTION HAVE TO SIGN AN INTELLECTUAL PROPERTY AGREEMENT DETERMINING WHO OWNS THE PATENTS. IT'S USUALLY THE UNIVERSITIES WHO OWN THE PATENTS WITH AN EXCLUSIVE LICENSE GOING TO.

THEY HAVE TO CERTIFY THAT THEY HAVE AN R&D ARRANGEMENT, AND VIRTUAL COMPANIES, AS I SAID BEFORE, DON'T QUALIFY. YOU NEED REAL COMPANIES WHO ARE GOING TO

PARTICIPATE IN THIS AND THAT MUST BE PROVEN AS WELL. AND THERE IS ALWAYS THE ISSUE OF CONFLICT OF INTEREST. IF YOU GET POST DOCS OR GRADUATE STUDENTS INVOLVED IN THE COMPANY RESEARCH, ARE YOU USING THEM AT LOW COST IN ORDER TO DEVELOP IT, AND THEN DO THEY BELONG ON THE PATENT. SO IT'S ALWAYS AN ISSUE. THE COMPANIES, THEY ALREADY SIGN OFF THEIR RIGHTS TO IT IN THE BEGINNING.

KEY THING IN THE NIH PROGRAM IS YOU CAN'T SWITCH BETWEEN THE TWO MECHANISMS BETWEEN PHASE I AND PHASE II. QUICKLY HOW THEY'RE REVIEWED AND AWARDED. THE SMALL BUSINESS OFTEN, IN FACT ALMOST ALWAYS, WORKS WITH AN ACADEMIC INSTITUTION IN AN INFORMAL ARRANGEMENT FOR SBIR'S OR A FORMAL ONE FOR STTR'S, AND THIS IS JUST THE SAME AS FOR OTHER NIH GRANTS. IT GOES OFF TO THE CENTER FOR SCIENTIFIC REVIEW WHERE IT GOES TO A PERSON WHO'S IN CHARGE OF THE REVIEW, AND THERE'S A REVIEW COMMITTEE THAT REVIEWS IT AND GIVES IT A MERIT SCORE AND A SUMMARY STATEMENT. IT GOES OFF TO THE INSTITUTE TO WHICHEVER PROGRAM IS GOING TO MANAGE THE APPLICATION, AND THE SUMMARY STATEMENT GOES BACK TO THE SMALL BUSINESS, THERE'S A SECONDARY REVIEW BY THE COUNCIL WITH THE INSTITUTE OR CENTER, AND GOES TO THE DIRECTOR, WHO RECOMMENDS EITHER FUNDING OR NOT FUNDING. AND IF THEY RECOMMEND FUNDING, THE GRANTS MANAGEMENT GRANT GETS AWARDED. EXACTLY THE SAME FOR ALL NIH GRANTS. WE DEAL

THE SAME WAY WITH BUSINESSES OR ACADEMIC GRANT APPLICATIONS.

AND THE REVIEW CRITERIA IS THE SAME AS WELL. NIH HAS THE SAME REVIEW CRITERIA FOR ALL APPLICATIONS. THERE'S THESE FIVE AREAS OF SIGNIFICANCE, THE APPROACH, THE INNOVATION, THE INVESTIGATOR, AND THE ENVIRONMENT. AND I DON'T NEED TO GO THROUGH THOSE, BUT THOSE ARE THE ONES THAT ARE DONE. THE NICE THING ABOUT THIS IS THE REVIEW COMMITTEES ARE USED TO USING THESE REVIEW CRITERIA. SO WE'RE NOT REINVENTING THINGS. WE DO HAVE ABOUT A THIRD OF THE MEMBERS ON THE REVIEW COMMITTEE COME FROM BUSINESS, SO THEY'RE LOOKING AT DO THESE PROJECTS ACTUALLY -- ARE THEY LIKELY TO END UP WITH PRODUCTS OR SERVICES THAT WILL BENEFIT PEOPLE, BUT THEY'RE USING JUST WHAT THEY KNOW HOW TO DO BEFORE.

I THINK THERE'S A BIG ADVANTAGE HERE IN NOT REINVENTING THE WHEEL EVERY TIME YOU'RE GOING TO DO A FUNDING.

SO THIS IS SOME DATA THAT YOU MIGHT BE INTERESTED IN TO GIVE YOU SOME IDEA HOW MANY OF THESE ACTUALLY GET AWARDED. THE SBIR'S ARE SOMEWHERE BETWEEN 16 PERCENT LAST YEAR, 19 PERCENT FOR THE STTR'S. I SAID THEY WERE A LITTLE BIT BETTER. AND YOU WILL NOTICE THAT FAST TRACKS ARE A LITTLE BIT LESS, AND THAT'S BECAUSE YOU HAVE TO HAVE TWO APPLICATIONS. IT'S HARDER TO GET THEM.

YOU WILL NOTICE THAT THE PHASE II'S HAVE A HIGHER PERCENT OF FUNDING, AND I THINK THAT'S BECAUSE YOU'VE ALREADY SELECTED THE BEST OF THE LOT FROM THE PHASE I'S, WHICH IS WHY I STRONGLY THINK IT'S IMPORTANT TO HAVE A PHASE I AND THEN A PHASE II EFFORT IN FUNDING BECAUSE YOU'RE GETTING THE BEST ONES TO APPLY AND GET THE PHASE I AWARDS, AND THEN YOU'RE LOOKING AT THOSE AND SEEING THE BEST ONES OF THOSE. SO YOU WOULD EXPECT THEM TO BE BETTER THAN YOU WOULD FOR PHASE I'S, THE PERCENTAGES TO BE BETTER. SO THERE'S THE FUNDING.

I THOUGHT YOU'D BE INTERESTED IN THIS. IT TAKES A FAIR AMOUNT OF WORK TO FIGURE OUT HOW YOU'RE GOING TO USE YOUR FUNDS.

CHAIRMAN PENHOET: RIGHT.

DR. MILMAN: AS YOU KNOW. HERE'S THE KEY THING.

DR. PIZZO: TAKES MORE WORK TO GET TO.

DR. MILMAN: FIRST YOU HAVE TO GET THE FUNDS AND THEN YOU CAN USE THEM. BUT YOU CAN ONLY GET PHASE II APPLICATIONS FROM PHASE I AWARDEES. AND YET THE PHASE II APPLICATIONS HAVE A LOT MORE MONEY INVOLVED, AND THEY ALSO HAVE MORE TIME. SO THE KINDS OF THINGS YOU HAVE TO THINK ABOUT, THIS PIE CHART SHOWS YOU FOR SBIR'S, THE PHASE I AWARDS, AND REMEMBER ONLY THE PHASE II'S CAN COME FROM THAT, WHICH ARE THESE ONES HERE, BUT SOME OF THE PHASE I AWARDS GET MORE THAN ONE YEAR, SO THERE'S SOME

OUT YEARS FOR THOSE. THESE ARE THE FAST TRACKS, WHICH ARE A SMALL PERCENTAGE, AND MOST OF THE PHASE II GET OUT YEARS FOR THAT. SO IF YOU FUND A LOT OF PHASE II'S -- REMEMBER, WE HAVE A FIXED AMOUNT OF MONEY, AND WE HAVE TO USE THE SAME AMOUNT OF MONEY ALL THE TIME. IF WE FUND TOO MANY PHASE II'S, WE HAVE NO PHASE I'S COMING UP, SO WE CAN'T HAVE PHASE II'S DOWN THE ROAD. WHAT YOU REALLY WANT TO DO, IN MY OPINION, IS HAVE MORE OF THESE ACTUALLY SO THAT YOU HAVE A BETTER CHOICE DOWN THE ROAD. SO THIS JUST GIVES YOU AN IDEA OF SORT OF WHAT WE DO, WE BEING NIH, IN TERMS OF THAT FUNDING CURVE. OKAY.

SO IN CONCLUSION, I'M GOING TO GIVE YOU WHAT I CONSIDER THE POINTS TO CONSIDER IF YOU WERE TO START A CIRM SMALL BUSINESS PROGRAM. OKAY. AND SO LET'S WALK THROUGH THESE AND WE'LL TAKE QUESTIONS ON THEM. THE FIRST IS DO YOU WANT TO HAVE BOTH AN SBIR AND AN STTR MECHANISM? ONE WHERE YOU FUND ONLY THE COMPANIES OR ONE WHERE YOU FUND COMPANIES TO DO RESEARCH WITH ACADEMIC INSTITUTIONS. I SUGGEST THAT BOTH ARE VERY VALUABLE.

WHAT PERCENT OF THE BUDGET WOULD YOU LIKE TO USE FOR THIS? WE KNOW WHAT NIH USES IS ABOUT 3 PERCENT OF THE TOTAL BUDGET, BUT YOU MIGHT DECIDE, SINCE YOUR GOAL IS TO HAVE MORE PRODUCTS PRODUCED, THAT YOU'D ACTUALLY LIKE TO INCREASE THAT BECAUSE YOU ARE GETTING A LOT OF LEVERAGE FROM THE SMALL BUSINESS PROGRAM COMPARED TO

FUNDING ACADEMIC INSTITUTIONS.

HOW MANY RECEIPT DATES WOULD YOU LIKE?

CHAIRMAN PENHOET: THE PERCENT AT THE FEDERAL LEVEL NOW IS DETERMINED BY STATUTE. SO IF YOU HAD OVERWHELMINGLY GOOD GRANTS IN THE SBIR CATEGORY --

DR. MILMAN: YOU'RE STILL STUCK.

CHAIRMAN PENHOET: -- AND A BUNCH OF LOUSY GRANTS IN RESEARCH --

DR. MILMAN: YOU CAN'T SWITCH.

CHAIRMAN PENHOET: -- CAN'T MOVE MONEY FROM ONE TO THE OTHER.

DR. MILMAN: WE CAN'T SWITCH. WE'RE DETERMINED BY STATUTE.

WE HAVE THREE RECEIPT DATES A YEAR. THE REASON BEING IS WHEN GOOD IDEAS COME UP, YOU'D LIKE TO SEE THE IDEAS AND YOU'D LIKE TO MAKE DECISIONS ON THEM, BUT IT'S A LOT OF WORK AND YOU HAVE TO DECIDE. OTHER AGENCIES HAVE ONLY A SINGLE RECEIPT DATE A YEAR. NSF, FOR EXAMPLE, HAS ONLY ONE. NSF, BY THE WAY, HAS SPECIFIC TOPICS THAT THEY'LL ACCEPT APPLICATIONS IN; WHEREAS, WE'LL ACCEPT APPLICATIONS IN ALL TOPICS. CLEARLY YOU WOULD ACCEPT APPLICATIONS ONLY IN STEM CELL RESEARCH, BUT YOU GET THE IDEA. YOU REALLY HAVE TO DECIDE HOW MANY TIMES WOULD YOU ALLOW THEM TO COME IN.

WHAT WILL BE YOUR REVIEW AND AWARD POLICIES AND

PROCEDURES? HOW WOULD YOU GO ABOUT SETTING IT SO YOU CAN EVALUATE THE VERY BEST GRANTS AND MAKE YOUR DECISIONS ON WHAT PERCENTAGE WOULD BE PHASE I AND WHAT PERCENTAGE WOULD BE PHASE II AND HOW MUCH DOLLARS TO PUT INTO IT? THESE ARE CONSIDERATIONS YOU NEED TO MAKE UP FRONT.

HOW LONG SHOULD THE TIME AND AWARD AMOUNTS BE? REMEMBER THE PHASE I FOR US IS MOSTLY ONE YEAR, BUT SOME ARE TWO YEARS. WOULD YOU ALLOW THAT? WHAT ABOUT THE PHASE II? HOW LONG WOULD YOU HAVE THOSE BE? HOW MUCH MONEY WOULD YOU PUT INTO IT? IF YOU DECIDE TO HAVE A FIXED PERCENTAGE OF THE CIRM BUDGET, THEN YOU HAVE TO ACTUALLY CAREFULLY DECIDE WHAT PERCENTAGE TO PUT INTO THESE.

WOULD YOU HAVE FAST TRACK? WOULD YOU ALLOW SOMEBODY TO COME IN AND APPLY FOR BOTH AT THE SAME TIME WHEN THEY HAVE A PROJECT THAT YOU KNOW IS GOING TO GO THROUGH, SAY, FOR FDA APPROVAL AND YOU JUST NEED TO KNOW THE MILESTONES THERE?

WHAT'S THE DEFINITION OF A SMALL COMPANY? WE HAVE 500 PEOPLE, BUT OUR DEFINITION IS MUCH MORE ON OWNERSHIP THAN IT IS ON PEOPLE.

WHAT'S THE DEFINITION OF RESEARCH AND DEVELOPMENT? WE ARE VERY FOCUSED ON RESEARCH, BUT YOU MIGHT WANT TO HAVE MORE FOCUS ON DEVELOPMENT. SO YOU'D SAY, OKAY, I'LL FUND BOTH TYPES, OR MAYBE YOU'D HAVE A

PERCENTAGE. YOU'D HAVE TO RESEARCH THE PERCENTAGE AND THE DEVELOPMENT.

WOULD YOU ALLOW VC COMPANY OWNERSHIP? THAT ALL DEPENDS ON WHETHER YOU REALLY WANT TO HAVE ECONOMIC DEVELOPMENT AS A PRIMARY GOAL HERE SO THAT YOU INCREASE THE NUMBER OF JOBS AND FUNDING IN THE STATE OF CALIFORNIA, OR DO YOU WANT TO HAVE PUBLIC HEALTH BENEFITS MORE, IN WHICH CASE YOU MIGHT TAKE THE COMPANIES THAT HAVE A BETTER CHANCE OF PUSHING IT FORWARD. SO THOSE ARE DECISIONS YOU NEED TO MAKE.

WOULD YOU WANT TO REQUIRE THAT THE PRINCIPAL PLACE OF BUSINESS BE IN CALIFORNIA AND NOWHERE ELSE?

WOULD YOU WANT TO REQUIRE THAT THE RESEARCH FUNDS BE USED TOTALLY IN CALIFORNIA? IS THIS THE PLACE THAT YOU CAN GET THE RESEARCH TO BE DONE AND ONLY BY CALIFORNIA COMPANIES IN CALIFORNIA? WOULD YOU WANT TO REQUIRE A MINIMUM PERCENTAGE OF EFFORT BY THE COMPANY? REMEMBER WE SAY THE COMPANY HAS TO DO A CERTAIN AMOUNT OF THE WORK. THEY CAN OUTSOURCE SOME OF IT, BUT WE WANT THEM TO BE REAL COMPANIES.

WOULD YOU WANT TO REQUIRE THAT THE PRODUCTS BE PRODUCED IN CALIFORNIA? SO WHAT YOU ARE GETTING OUT OF IT IS ACTUALLY CALIFORNIA ECONOMIC DEVELOPMENT. WHAT ABOUT MARCH-IN RIGHTS? YOU WANT TO HAVE THE ABILITY TO TAKE OVER THE PATENTS OF SOMETHING IF THE COMPANIES DON'T

WANT TO PURSUE THEM? AND WHAT ABOUT INTELLECTUAL PROPERTY AGREEMENTS AND LICENSES? DO YOU WANT TO HAVE A ROYALTY FREE LICENSE TO PRACTICE WHATEVER THE PATENT IS?

SO THAT'S A SUMMARY OF WHAT WE DO IN THE SMALL BUSINESS PROGRAMS. WHEN I TALKED TO ED, I SAID THERE ARE A LOT OF OTHER TOPICS YOU MIGHT BE INTERESTED IN. WE DON'T HAVE TIME FOR THEM TODAY, BUT A FEW OF THEM ARE HERE. I DEVELOPED THE NIH AIDS REAGENT PROGRAM, WHICH WE USED TO PROVIDE REAGENTS IN A CENTRAL SOURCE SO THAT COMPANIES COULD ACTUALLY SHARE THEM. YOU KNOW, THE DIFFICULTY COMPANIES HAVE SHARING REAGENTS IS THE LAWYERS GET INVOLVED, AND THEY NEVER WANT TO TALK TO EACH OTHER, AND IT CAN TAKE LITERALLY YEARS TO GET SOMETHING TO TAKE PLACE. WE SET UP A PROGRAM WHERE PEOPLE DONATED THE REAGENTS, AND THEY WERE ABLE TO TAKE REAGENTS OUT, AND THERE ARE A LOT OF LEGAL REQUIREMENTS ON WHAT YOU CAN DO WITH THEM. THAT MIGHT BE VERY INTERESTING TO YOU IN TERMS OF WHAT YOU ARE GOING TO DEVELOP.

THE OTHER THAT YOU MIGHT BE INTERESTED IN LEARNING ABOUT IS OUR CENTERS FOR AIDS RESEARCH, WHICH WAS A WAY OF DEVELOPING COLLABORATIVE CENTERS THAT WORKED ON PROJECTS. SO FROM THE GET-GO, THEY WERE ACTUALLY WORKING TOGETHER.

SO THOSE ARE THINGS FOR THE FUTURE IF YOU'RE INTERESTED. AND IF YOU NEED TO CONTACT ME, THIS IS MY

CONTACT INFORMATION. I'D BE HAPPY TO TALK TO ANYBODY FURTHER. I THINK I'VE GOT ABOUT FOUR MINUTES FOR QUESTIONS.

CHAIRMAN PENHOET: THANK YOU. THANK YOU VERY MUCH.

(APPLAUSE.)

MR. GOLDBERG: THAT WAS AN OUTSTANDING PRESENTATION. I LEARNED A LOT MORE THAN I EXPECTED TO. DO YOU HAVE ANY SENSE -- THIS HAS UNQUESTIONABLY BEEN AN EXTREMELY EFFECTIVE PROGRAM FOR THE GOVERNMENT, BUT IT'S GONE ON SINCE 1982. SO THE DURATION OF BENEFIT MAY OR MAY NOT HAVE BEEN SIMULATED IN THE FIRST HALF, THE SECOND HALF EQUALLY OVER THE COURSE OF THE ENTIRE PERIOD. AS YOU KNOW, OUR FUNDING PERIOD IS APPROXIMATELY TEN YEARS. IT WOULD BE HELPFUL TO KNOW IF YOU COULD -- IF YOU DO KNOW OR IF YOU COULD MASSAGE THE DATA TO ADDRESS THIS, WHAT THE FIRST 10-YEAR IMPACT WAS IN TERMS OF THOSE PERFORMANCE METRICS WHICH YOU IDENTIFIED.

DR. MILMAN: SO THE QUESTION IS HOW WELL DID THE FIRST TEN YEARS OF THE PROGRAM WORK IN TERMS OF PRODUCING PRODUCTS OR SERVICES THAT BENEFIT HEALTH? AND THE NATIONAL ACADEMY HAS DONE A PROJECT ON THAT. I'M NOT SURE, IN MY OPINION, THAT THEY CAN ACTUALLY MEASURE IT EFFECTIVELY, WHICH IS WHY I SET OUT TO GIVE YOU SURROGATES FOR SUCCESS. AND THE REASON IS THAT THESE

COMPANIES, CERTAINLY THEN AND TODAY, MOST OF THEM DO NOT END UP BEING STANDALONE COMPANIES THAT CONTINUE TO EXIST AND MAKE PRODUCTS. THE GOAL TO ME EXIT STRATEGY IN MOST COMPANIES TODAY IS TO BE ACQUIRED BY A BIGGER COMPANY. AND FOLLOWING THAT ALONG IS VERY DIFFICULT BECAUSE YOU CAN'T SAY, WE PUT IN, AS I INDICATED, MAYBE A FEW MILLION DOLLARS INTO A PROJECT, BUT IF IT'S GOING TO TAKE 500 MILLION OR MORE TO DO IT, CLEARLY IT'S GOING TO DEPEND ON WHO THEY GET ACQUIRED BY AND WHAT HAPPENS AFTERWARDS.

SO I THINK THE SURROGATES ARE BETTER. MY RECOMMENDATION TO YOU WOULD BE TO USE SOME OF THESE SURROGATES, IF YOU ARE FUNDING COMPANIES, TO SEE ARE WE GETTING MATCHING VC FUNDS, ARE THEY GETTING PATENTS, HOW IS IT WORKING, ARE THEY HITTING THEIR MILESTONES BECAUSE THE OTHER PROBLEM OF ACTUALLY SEEING WHETHER THEY'RE SUCCESSFUL IS SOMETHING THAT'S VERY DIFFICULT TO MEASURE.

DR. STEWARD: JUST FOLLOWING UP ON THAT, LOOKING BACK, IS THERE SOMETHING THAT COULD HAVE BEEN PUT IN PLACE IN THE BEGINNING THAT WOULD HAVE MADE THAT ACCOUNTABILITY EASIER?

DR. MILMAN: WELL, WE KNOW ABOUT THE PATENTS BECAUSE THEY'RE REQUIRED TO TELL US ABOUT THE PATENTS. AND THE PROBLEM IS ACTUALLY FOLLOWING WHAT PRODUCTS COME OUT OF THOSE PATENTS. AND I THINK A LOT OF IT'S BEEN DONE NOT BY THE GOVERNMENT, WHICH IS WHY I MADE THE

LITTLE PLEA THERE. IF YOU'D LIKE ADDITIONAL INFORMATION, I'D GO TO ANN ESKESEN AT INKNOWVATION BECAUSE SHE FOLLOWS ALL THIS IN TERMS OF WHAT COMES OUT OF THESE COMPANIES, NOT IN JUST HEALTH, BUT ACROSS THE WHOLE OF THEM. AND HER BIG CLAIM IS THAT WHAT REALLY COMES OUT OF THESE COMPANIES IS INTELLECTUAL PROPERTY, AND THAT'S WHERE THE VALUE IS. I THINK THAT'S A KEY THING IN GENERAL. IT'S NOT THE PRODUCTS; IT'S THE IP AND HOW IT'S USED TO ACTUALLY MAKE PRODUCTS DOWN THE ROAD THAT'S REALLY CRITICALLY IMPORTANT.

ONE OF THE CRITICAL THINGS AGAIN, AND I WANT TO EMPHASIZE, ABOUT THE GOVERNMENT SMALL BUSINESS PROGRAM, I THINK LEAVING THE IP WITH THE COMPANIES MAKES THE COMPANIES SUCCESSFUL AT GETTING LEVERAGE TO MOVE FORWARD AND SELLING THAT IP DOWN THE ROAD.

DR. PIZZO: GREG, FOR THE STTR PROGRAM IN RELATIONSHIP TO ACADEMIC INSTITUTIONS, ARE THERE SITUATIONS WHERE THE ACADEMIC INSTITUTION OR INVESTIGATOR CHOSE, OR YOU MAY NOT KNOW THIS, BUT WHERE THEY CHOSE NOT TO GO THROUGH THE ROUTE OF STTR, BUT STILL WENT A COMMERCIALIZATION ROUTE? IF SO, DO SOME VIEW THAT AS MORE BENEFICIAL, MORE FINANCIALLY SUCCESSFUL?

DR. MILMAN: SO THE QUESTION IS DO ACADEMIC INVESTIGATORS PREFER THE STTR ROUTE COMPARED TO AN SBIR ROUTE? CAN I REPHRASE IT THAT WAY?

DR. PIZZO: OR NEITHER.

DR. MILMAN: OR NEITHER. I CAN'T TELL ABOUT IF THEY GO A DIFFERENT WAY. I REALLY DON'T KNOW.

DR. PIZZO: I WAS REALLY THINKING ABOUT PEOPLE JUST BYPASS THE WHOLE THING.

DR. MILMAN: I THINK THAT MANY ACADEMIC INVESTIGATORS, THE OUTSTANDING ONES, THE HOWARD HUGHES INVESTIGATORS AND THE OTHERS THAT ARE VERY WELL KNOWN, ARE TRACKED VERY WELL BY THE PHARMACEUTICAL COMPANIES WHO COME IN AND MAKE AGREEMENTS AND LICENSING BOTH WITH THEM AND WITH THEIR INSTITUTIONS UP FRONT SO THAT THEY ACTUALLY DON'T GO THROUGH PERHAPS THE SMALL BUSINESS PROGRAM.

WHAT HAPPENS IS THOSE WHO DECIDE TO START THE COMPANIES ON THEIR OWN USE THIS AS A WAY OF GETTING FUNDING TO ACTUALLY START.

DR. PIZZO: SO MAYBE ASK THE QUESTION A DIFFERENT WAY. YOU'VE GOT DATA IN CALIFORNIA. IF YOU LOOK AT THE SORT OF MORE ENTREPRENEURIAL ACADEMIC INSTITUTIONS, WHICH I THINK OURS WOULD COUNT AS ONE, I DON'T KNOW ABOUT LOT OF ACTIVITY THAT'S GOING ON BY OUR FACULTY USING STTR OR SBIR. I COULD BE -- IT MAY JUST BE OFF MY RADAR SCREEN. YOU WOULD KNOW THAT.

DR. MILMAN: I DON'T KNOW IT PERSONALLY, BUT IT'S EASY TO FIND OUT. IT'S NOT HARD AT ALL TO FIND OUT.

DR. PIZZO: I THINK IT MIGHT BE INTERESTING AS AN EXERCISE TO FIND OUT FROM PLACES THAT DO HAVE A LOT OF COMMERCIAL ACTIVITY WHETHER THERE'S A LACK OF UTILIZATION OR A REASON WHY UTILIZATION MECHANISM IS NOT --

DR. MILMAN: LET ME ANSWER IT THIS WAY. IT'S BEEN AWHILE SINCE I'VE DONE THIS, BUT I'VE LOOKED AT THE ZIP CODES OF WHERE COMPANIES ARE THAT GET THESE FUNDING COMPARED TO THE ZIP CODES OF ACADEMIC INSTITUTIONS. WHAT YOU FIND, AND THE REASON THAT CALIFORNIA DOES SO WELL IN THE SBIR, STTR PROGRAM IS THE COMPANIES ARE CIRCULAR AROUND ACADEMIC INSTITUTIONS, SO THERE ARE LOTS OF THEM IN CALIFORNIA.

DR. PIZZO: 94305.

DR. MILMAN: THEY DO THAT. MY INTERPRETATION IS THAT COMPANIES START UP NEAR THEM BECAUSE I KNOW AS AN ACADEMIC FACULTY MEMBER, WHEN ED AND I WERE TOGETHER --

DR. GOLDSTEIN: THIS IS BOB GOLDSTEIN.

DR. MILMAN: HI, BOB. THIS IS GREG MILMAN.

CHAIRMAN PENHOET: GREG IS FINISHING UP, BOB, SO IF YOU DON'T MIND, WE'LL BE WITH YOU IN A FEW MINUTES.

DR. GOLDSTEIN: NO PROBLEM. THANK YOU.

DR. PRIETO: THIS IS FRANCISCO PRIETO. I'VE BEEN ONLINE FOR A LITTLE BIT HERE.

DR. WRIGHT: JANET WRIGHT ALSO.

CHAIRMAN PENHOET: WE NOW HAVE A QUORUM.

DR. MILMAN: I'VE LOST MY TRAIN OF THOUGHT. WHAT WERE WE TALKING ABOUT?

CHAIRMAN PENHOET: ZIP CODES.

DR. MILMAN: WHERE THE COMPANIES WERE. WHAT I WAS GOING TO TELL YOU IS WHEN ED AND I WERE IN THE BIOCHEMISTRY DEPARTMENT AT BERKELEY, IT WAS HARD TO WALK UP TO THE MOLECULAR BIOLOGY DEPARTMENT. AND ACADEMIC PEOPLE, BECAUSE YOU DON'T WANT TO GO THAT FAR, DON'T WANT TO GO VERY FAR TO WORK WITH COMPANIES. SO THE COMPANIES USUALLY SURROUND THE UNIVERSITIES. MANY UNIVERSITIES HAVE TAKEN UP SETTING UP INCUBATORS RIGHT ON THEIR SITES. IT WORKS WELL. WHEN THEY DO, THEY FIND THAT THESE ACADEMIC PEOPLE GO IN AND START THE COMPANIES AND THE INCUBATORS. IT'S A VERY POPULAR WAY OF GETTING IT DONE.

WHAT I DID FIND INTERESTING, YOU CAN ACTUALLY LOOK THE RATIO OF FUNDING OF SMALL BUSINESS GRANTS TO ACADEMIC GRANTS BECAUSE IT SHOULD BE ABOUT 2.8 PERCENT, RIGHT. ACTUALLY IT'S HIGHER THAN THAT IN CALIFORNIA, MEANING THAT CALIFORNIA ACTUALLY STARTS MORE COMPANIES WITH SBIR'S THAN OTHER PLACES. INTERESTINGLY, IN MY OPINION, IT'S LOW IN PENNSYLVANIA AND NEW JERSEY BECAUSE THE BIG PHARMACEUTICAL COMPANIES ARE THERE AND THEY ALREADY HAVE AGREEMENTS WITH THE ACADEMIC PEOPLE. SO THE ACADEMICS DON'T HAVE AN INCENTIVE TO GO OUT AND START COMPANIES. THAT'S JUST AN ASIDE HERE.

CHAIRMAN PENHOET: WE HAVE QUESTIONS FROM ANY OF OUR REMOTE LOCATIONS? FRANCISCO PRIETO AT SUTTER MEDICAL?

DR. PRIETO: YES.

CHAIRMAN PENHOET: JANET WRIGHT IN CHICO?

DR. WRIGHT: NOPE.

CHAIRMAN PENHOET: JOHN REED AT BURNHAM? IRVINE?

DR. REED: NO, NO ONE AT BURNHAM.

DR. BRYANT: SUE BRYANT HERE AT IRVINE.

CHAIRMAN PENHOET: DO YOU HAVE A QUESTION?

DR. BRYANT: NO.

CHAIRMAN PENHOET: IN LOS ANGELES?

DR. FONTANA: NO QUESTIONS.

CHAIRMAN PENHOET: HOW ABOUT FROM OUR ASSEMBLED AUDIENCE HERE IN SAN FRANCISCO? ANY QUESTIONS OF DR. MILMAN AT THIS POINT?

DR. PREMACK: I'M BRETT PREMACK FROM CHEMOCENTRYX. AND I'VE HAD SBIR'S, DOD, DARPA, NIH GRANTS. IT'S OFTEN THE CASE THAT THE SBIR FAVORS VERY SMALL COMPANIES BASED ON THE VENTURE CAPITAL STRUCTURE AND SOME OF THE OTHER DEFINITIONS. FOR THE STEM CELL RESEARCH PART, YOU MAY NOT WANT TO OVER TEN YEARS, AS YOU BROUGHT UP, START FUNDING AND MAKING COMPANIES BECAUSE YOU WANT TO GET PRODUCTS DONE QUICKLY. WE HAVE NOT

FAVORED THE SBIR MECHANISM BECAUSE THE PHASE I, PHASE II STARTS WITH A VERY SMALL AMOUNT OF MONEY, $100,000 OR SOMETHING OVER SIX MONTHS. YOU CAN'T BUDGET ON GETTING THE SECOND HALF. IT'S HARD TO MOVE FORWARD, HARD TO PLAN FOR THE FUTURE. IT'S REALLY FOR PEOPLE THAT HAVE AN IDEA, SPECIFICALLY A TECHNOLOGY IDEA LARGELY, A PLATFORM IDEA, RATHER THAN A THERAPEUTIC IDEA, AND THEY'RE MOVING THOSE IN TIMELINES THAT I DON'T THINK ARE CONSISTENT WITH WHAT WE NEED TO DO IN THE STATE, A FIXED TIMELINE, A CERTAIN BAG OF MONEY, CERTAIN PERIOD OF TIME.

THERE ARE OTHER NIH MECHANISMS THAT MOVE MUCH MORE QUICKLY. AND ONE OF THE BEST PROBABLY IS THE U 19 MECHANISM. THEY CALL IT A COOPERATIVE AGREEMENT. IT'S HALFWAY BETWEEN A GRANT AND A CONTRACT. EVERY THREE MONTHS YOU SEND IN A PROGRESS REPORT. UNLIKE AN NIH GRANT, THE PROGRESS REPORT COMES QUARTERLY. YOU MOVE VERY QUICKLY, AND THE MONEY IS FLUID. IT CAN GO UP DURING THE PROGRESS SEEN IN THAT PROGRAM.

IT'S A LITTLE TRICKY TO BUDGET, BUT IT HAS THE ADVANTAGE THAT YOU KNOW, AS A COMPANY, THAT YOU'VE GOT THIS FOR A CERTAIN NUMBER OF YEARS. YOU'RE NOT APPLYING FOR A HUNDRED K FOR SIX MONTHS. THAT WAS THE FIRST THING.

THE SECOND THING I WANTED TO SAY IS THAT OVERALL THE NIH DEFINES RETURN ON INVESTMENT VERY DIFFERENTLY

THAN CIRM. SO THEY DEFINE RETURN ON INVESTMENT AS TAX DOLLARS SAVED BASICALLY DUE TO HEALTHCARE RESEARCH. I THINK BEFORE YOU CAN TALK -- YOU MENTIONED SOME IDEAS ABOUT ROYALTIES OR WHETHER THERE'S ROYALTY FEE IN RESEARCH. YOU HAVE TO DECIDE HOW YOU'RE GOING TO DEFINE RETURN ON INVESTMENT BECAUSE THE NIH HAS SET UP WHERE RETURN ON INVESTMENT IS ALREADY DEFINED ONLY AS SAVINGS IN HEALTHCARE DOLLARS. THAT'S A LITTLE DIFFERENT THAN WHAT WE'RE THINKING HERE.

DR. MILMAN: ACTUALLY I DON'T KNOW ANYTHING ABOUT THAT RETURN ON INVESTMENT. IN MY 18 YEARS THERE AT NIH, WE NEVER TALKED ABOUT RETURN ON INVESTMENT AS HEALTHCARE DOLLARS.

DR. PREMACK: IT'S IN THE BAYH-DOLE ACT THOUGH.

DR. MILMAN: IT MAY BE IN IT, BUT IT'S NEVER USED. THERE ARE A LOT OF THINGS THAT ARE IN WRITING THAT AREN'T TRUE. I CAN GIVE YOU AN EXAMPLE OF ONE OF THEM. IT SAYS IN THE SMALL BUSINESS ADMINISTRATION, THE SBIR SOLICITATION, THAT IN ORDER TO APPLY FOR AN NIH SBIR-STTR GRANT, YOU DON'T NEED ANY PRELIMINARY DATA. WHAT I ALWAYS TELL PEOPLE IS IT'S ABSOLUTELY TRUE YOU DON'T NEED ANY PRELIMINARY DATA TO APPLY FOR A GRANT. YOU DO NEED PRELIMINARY DATA TO GET FUNDED THOUGH. SO YOU CAN APPLY ALL YOU WANT, BUT YOU WON'T GET ANY MONEY.

I THINK WHAT YOU'RE SAYING IS ONE OF THOSE

THINGS THAT MAY BE IN WRITING, BUT IT'S NEVER USED. IN TERMS OF AMOUNT OF DOLLARS, I POINTED OUT WHAT THE GUIDELINES ARE, BUT YOU WILL NOTICE THAT THE MEDIAN AMOUNT IS WAY ABOVE THE GUIDELINES. IN FACT, FOR OTHER PROGRAMS, IN MY INSTITUTE WE HAVE AN ADVANCED TECHNOLOGY PROGRAM THAT'S $300,000 A YEAR FOR UP TO TWO YEARS FOR PHASE I AND A MILLION DOLLARS A YEAR UP TO THREE YEARS FOR PHASE II, AND WE ACTUALLY HAD A BIODEFENSE ON, THERE WAS AN IMPORTANT THING TO GET SOMETHING DONE, OF HALF A MILLION DOLLARS A YEAR PER YEAR FOR TWO YEARS FOR PHASE I AND $2 MILLION A YEAR FOR THREE YEARS FOR PHASE II. SO THE AMOUNT OF DOLLARS YOU CAN TWEAK DEPENDING UPON WHAT YOUR GOALS ARE AND SPECIFICALLY WHAT THEY'RE DOING.

THE KEY THING, I THINK, ABOUT THIS PROGRAM THAT'S OF VALUE IS THAT YOU START OUT WITH PHASE I WITH A PROOF OF CONCEPT, AND THEN YOU MOVE FORWARD TO FUNDING OF THINGS IF THERE ARE MILESTONES.

CHAIRMAN PENHOET: ANY OTHER COMMENTS FROM THE PUBLIC? WE'RE GOING TO HEAR SOME MORE FROM COMPANIES TODAY WHO HAVE UTILIZED MANY OTHER PROGRAMS IN THE GOVERNMENT FOR FUNDING, SO WE'LL LEARN MORE ABOUT THOSE. WITH THAT, THANK YOU VERY MUCH, GREG --

(APPLAUSE.)

CHAIRMAN PENHOET: -- FOR COMING OUT HERE FROM WASHINGTON TO SEE US.

ON THE PHONE NOW WE HAVE THE NEXT SPEAKER, ROBERT GOLDSTEIN. ROBERT IS THE CHIEF SCIENTIFIC OFFICER OF THE JUVENILE DIABETES RESEARCH FOUNDATION INTERNATIONAL. WE'VE ASKED ROBERT TO SPEAK BECAUSE THE FUNDING OF COMPANY PROGRAMS BY NONPROFIT ORGANIZATIONS SUCH AS JDRF IS A RELATIVELY NEW PHENOMENON AND, AS I UNDERSTAND IT, HAS BEEN DRIVEN BY MANY OF THESE ORGANIZATIONS' BELIEF THAT THEY CAN MOVE THEIR INTERESTS, THAT IS, FOR BETTER THERAPIES, BETTER TREATMENT GENERALLY OF PATIENTS WHO THEY REPRESENT. THEY CAN DO IT MORE EFFECTIVELY BY FUNDING PROGRAMS SOMETIMES IN COMPANIES THAN WHAT'S TRADITIONALLY BEEN DONE IN THE NONPROFIT SECTOR. JDRF IS A LEADER IN THE FIELD OF JUVENILE DIABETES RESEARCH, ETC.

ROBERT, WE'RE VERY PLEASED TO HAVE YOU JOIN US TODAY AND GIVE US YOUR PERSPECTIVE ON HOW JDRF THINKS ABOUT FUNDING FOR-PROFIT ORGANIZATIONS.

DR. GOLDSTEIN: THANK YOU VERY MUCH. DO YOU HAVE ACCESS TO MY SLIDES?

DR. MAXON: DID YOU SEND SOME?

MS. KING: WE'RE WONDERING DID YOU E-MAIL THOSE SLIDES TO DR. MAXON? SHE'S LOOKING FOR THEM POTENTIALLY IN HER E-MAIL RIGHT NOW, BUT WE'VE BEEN HERE FOR A WHILE.

DR. GOLDSTEIN: I'M SORRY. THEY WERE E-MAILED LATE YESTERDAY. BUT WE CAN DO IT WITHOUT SLIDES. I

DON'T WANT TO WASTE YOUR TIME.

CHAIRMAN PENHOET: FOR SOME REASON THEY DIDN'T COME THROUGH. WE'RE SORRY ABOUT THAT. IF YOU WOULDN'T MIND, YOU COULD JUST GIVE US A VERBAL DESCRIPTION OF YOUR EXPERIENCE. AND IF WE GET THE SLIDES, WE WILL CIRCULATE THEM TO THE GROUP.

DR. GOLDSTEIN: FIRST OF ALL, I NEED TO MAKE A COMMERCIAL ANNOUNCEMENT THAT THE JDRF MISSION IS TO FIND A CURE FOR TYPE 1 DIABETES. AND TO ADDRESS THAT ISSUE, WE FUND NOT-PROFIT AND FOR-PROFIT ORGANIZATIONS EITHER IN THE UNITED STATES OR OUTSIDE. ABOUT 38 PERCENT OF OUR $100 MILLION SPEND THIS YEAR WENT OUTSIDE THE UNITED STATES. AND THAT GIVES US, IN CONTRAST TO MANY FOUNDATIONS, A KIND OF UNIQUE INTERNATIONAL EXPERIENCE WHICH IS QUITE RELEVANT IN THE SCIENTIFIC WORLD OF STEM CELL RESEARCH.

THE OTHER REASON FOR INTRODUCING OUR CORE PRINCIPLES, WHICH INCLUDE THE COMMITMENT TO DISSEMINATE INFORMATION, SHARE RESOURCES, ETC., IS THAT WE STRIVE FOR A PUBLIC MODEL. AND SO THE INTELLECTUAL PROPERTY POLICY, IF YOU WILL, REALLY STARTS WITH OUR INTELLECTUAL PROPERTY POLICY FOR NOT-PROFIT ORGANIZATIONS. AND ALTHOUGH IT'S A TINY BIT REPETITIVE IN THAT CALIFORNIA HAS A VERY NICE 38-PAGE SUMMARY OF ITS POLICIES FOR NOT-PROFITS, JUST A FEW HIGHLIGHTS TO POINT.

WE LET THE IP REMAIN WITH THE GRANTEE ORGANIZATION. WE ASK THEM TO FILE INFORMATION ABOUT PATENTS AND INVENTIONS. WE DO NOT PAY FOR PATENT FILING. IF PEOPLE ABANDON THAT POSSIBILITY, WE RESERVE MARCH-IN RIGHTS. OUR BASIC FUNDAMENTAL ASK FOR NOT-PROFITS IS THAT WE'LL SHARE AT SOME FUTURE DATE IN THE MONEY STREAM IF SOMETHING SUCCEEDS. WE DON'T ACTUALLY TRY TO SEEK OWNERSHIP PER SE. WE PROTECT THE FIRST 250 OR $500,000 WORTH OF SO-CALLED PROFITS SO WE CAN REINVEST IN RESEARCH. AND THE POLICY IS INTENDED TO ENCOURAGE THE DISSEMINATION OF INFORMATION, EFFICIENT UTILIZATION OF DISCOVERIES. AND WE MAKE IT OR WE HOPE TO MAKE IT SO IT DOESN'T ACT AS AN IMPEDIMENT TO PROGRESS.

THE INTELLECTUAL PROPERTY MODELS THAT ARE OUT THERE ARE FAIRLY SIMILAR AMONGST THE MEDICAL RESEARCH COUNCILS OF THE WORLD AND, FOR EXAMPLE, THE WELLCOME TRUST WHERE MOST OF THE NOT-PROFIT VERSION RESIDES IN THE GRANTEE RECIPIENTS. ALTHOUGH THE WELLCOME TRUST, AS POINT OF INFORMATION, SEVERAL YEARS AGO ESTABLISHED ITS OWN FOR-PROFIT ARM CALLED CATALYST, WHICH PROVIDED FUNDS TO COMPANIES FOR DEVELOPMENT PURPOSES, THEY CHANGED ITS INDEPENDENCE AND BROUGHT IT BACK WITHIN THE TRUST. IT WASN'T SO EASY TO ADMINISTER. FOR PEOPLE ENGAGED IN PROGRAMS, SPEAKING TO THE WELLCOME TRUST ABOUT THEIR GOOD AND BAD EXPERIENCES MAY BE HELPFUL OR VALUABLE.

NOW, WE INITIALLY CREATED AN OPPORTUNITY TO FUND GRANTS TO INDUSTRY. WE CALLED IT OUR INDUSTRY DISCOVERY AND DEVELOPMENT PROGRAM. AND IN ITS EARLY RENDITION WAS NOT UNLIKE THE SBIR PROGRAM, BUT THE IDEA WAS TO OPEN THE DOOR TO COMPANIES THAT COULD DO INTERESTING AND USEFUL THINGS TO HELP OUR AGENDA. AND THAT PROGRAM IS INTENDED TO FOSTER COLLABORATION BETWEEN US AND INDUSTRY PARTNERS. THE COMPANIES THAT WE FUND TENDED TO BE SMALL COMPANIES IN PART BECAUSE THE KIND OF PROOF OF CONCEPT IDEAS ARE FROM EARLY DISCOVERY WORK AND NOT LATE STAGE DEVELOPMENT ACTIVITIES.

WE INSIST THAT THE COMPANIES HAD SUFFICIENT RESOURCES TO EITHER MATCH WHAT WE WERE DOING OR STAY ALIVE. SO WE DID SOME SORT OF DUE DILIGENCE FROM A BUSINESS POINT OF VIEW ON AN INDUSTRY GRANT. BUT THE INDIVIDUAL CONTRACTS ARE NEGOTIATED ON A CASE-BY-CASE BASIS AND INVOLVE A VARIETY OF AGREEMENTS. BUT THE BASIC INTELLECTUAL PROPERTY ISSUES, WE RELIED ON THE SAME KIND OF PRINCIPLES. IN OTHER WORDS, WE DIDN'T WANT THE COMPANIES TO KEEP INFORMATION A SECRET FOREVER. SO WE HAVE RANGING FROM SIX MONTHS TO 12 MONTHS ABILITY TO SLOW DOWN PUBLICATION, BUT NOT FOREVER.

IN THE INDIVIDUAL ONE-ON-ONE NEGOTIATIONS WITH COMPANIES, THE EASIEST THING AND THE ITEM THAT PRODUCES THE SMALLEST IMPEDIMENT IS ACTUALLY ASKING FOR MONEY AS

OPPOSED TO OWNERSHIP OR PERCENTAGES, ETC. AND THAT'S, I THINK, A RECURRING THEME OF WHAT COMPANIES DO OR DO NOT WISH TO HAVE.

WE'VE ACTUALLY HAD SOME COMPANIES WHO HAVE NOT ACCEPTED OUR MONEY BECAUSE OF OUR DESIRE TO SHARE INFORMATION AND PUBLICLY DISCLOSE FINDINGS AT A CERTAIN POINT IN TIME. THINGS THAT MOST COMPANIES ACCEPT, SOME DON'T LIKE. BUT THE ULTIMATE MAIN ACTIVITY IS TO SHARE INCOME FROM FUTURE PROFITS. AND WE ESTABLISH SEPARATE OVERSIGHT TYPICALLY ON A SIX-MONTH BASIS IN TERMS OF FOLLOWING UP MILESTONES AND THOSE KIND OF ISSUES.

THE NEGOTIATION AND EXECUTION PERIOD TO GET A GRANT TO A COMPANY IS TWO OR THREE TIMES -- HAS TAKEN US TWO OR THREE TIMES AS LONG AS IT DOES TO NEGOTIATE OR PREPARE A CONTRACT WITH A UNIVERSITY BECAUSE FOR WHATEVER REASON THERE ARE LAYERS AND LAYERS OF LAWYERING THAT GETS INVOLVED. AND THE OTHER ISSUE WITH OUR RELATIONSHIP WITH WHAT I WOULD CALL SELECTED COMPANIES, PARTICULARLY WHERE STEM CELL RESEARCH IS CONCERNED, IS THAT THERE'S BEEN NO UNIFORM APPROACH AS FAR AS WE CAN TELL TO ETHICAL OVERSIGHT AND REVIEW. SO WE FIND VARYING COMPANY SOLUTIONS RANGING FROM COMPANIES ESTABLISHING THEIR OWN, LET'S SAY, STEM CELL OVERSIGHT COMMITTEE TO RESPOND TO ETHICS TO COMPANIES USING THEIR LOCAL UNIVERSITY ETHICAL OVERSIGHT TO COMPANIES ASKING US IF WE CAN DO WITH OUR

OVERSIGHT COMMITTEE. SO THE LACK OF A NATIONAL POLICY ON THAT KIND OF THING TURNS OUT NOT TO BE VERY HELPFUL.

AND YOU MAY NOT HAVE THE SAME ISSUE BECAUSE I WOULD ASSUME THAT MOST OF YOUR MONEY IS GOING TO STAY WITHIN THE BORDERS OF CALIFORNIA, SO YOUR ETHICS COULD BE EASIER TO ORGANIZE.

ISSUES OF CONFIDENTIALITY, NONDISCLOSURE, AND THOSE KIND OF THINGS REPRESENT, I DON'T KNOW, MAJOR PROBLEMS, I WOULD CALL THEM MINOR PROBLEMS, BUT LIABILITY AND INDEMNIFICATION TURN OUT TO BE A LITTLE MORE MAJOR PROBLEM BECAUSE, AS YOU CAN IMAGINE, OUR BOARD OF DIRECTORS, SORT OF PATIENT ADVOCATES FOR TYPE 1 DIABETES, DON'T WANT TO BE HELD LIABLE OR RESPONSIBLE FOR STUDIES THAT SOMEHOW GO AWRY IN ANY PARTICULAR FASHION. SO WE TYPICALLY ASK -- IT'S EASY FOR US WITH A UNIVERSITY BECAUSE THE UNIVERSITY IS ASSUMING THE RESPONSIBILITY. IT'S A LITTLE HARDER FOR LIKE A YOUNG SMALL COMPANY TO UNDERSTAND HOW EASILY YOU CAN WRESTLE INDEMNIFICATION AND SUFFICIENT LIABILITY ISSUES EVEN THOUGH YOU DON'T NECESSARILY KNOW WHAT YOU'RE THINKING ABOUT EVEN IF YOU ARE NOT THINKING ABOUT ANY KIND OF BAD POSSIBILITIES.

SO THE TAKE ON EXECUTING CONTRACTS OR GRANTS OR WHATEVER YOU WANT TO CALL THEM FOR FOR-PROFITS IS, I WOULD SAY, THE MAGNITUDE OF THE TEDIOUSNESS AND THE LAWYERING IS FIVE TO TEN X WHAT IT IS FOR YOUR KIND OF

STANDARD NOT-FOR-PROFIT UNIVERSITY-TYPE NEGOTIATION. THAT DOESN'T MEAN WE DON'T WANT TO DO IT. IT JUST MEANS YOU PAY A LOT OF ATTENTION TO THAT.

WHERE THAT BECOMES ADDITIONALLY INTERESTING AND MAY OR MAY NOT AFFECT YOU DIRECTLY, ALTHOUGH I WANTED TO RAISE IT AS A TOPIC, IS WHEN YOU HAVE RESEARCH COLLABORATIONS WITH OTHERS BEYOND YOUR BORDERS, WHETHER IN THE UNITED STATES OR OUTSIDE THE UNITED STATES, AND I'M GOING TO COME BACK TO WHAT I WOULD CALL THE CONSEQUENCES OF COLLABORATING EITHER OUTSIDE CALIFORNIA OR IN EUROPE OR SOME OTHER PLACE ON EARTH AND THE KIND OF DIFFICULTIES OR POTENTIAL PROBLEMS THAT THAT RAISES.

THE OTHER PART ABOUT COLLABORATION WHICH MAY OR MAY NOT BE AN ISSUE IS WE HAVE, FOR EXAMPLE, A STEM CELL PARTNERSHIP IN EUROPE THAT INVOLVES A CONTRIBUTION FROM A MAJOR PHARMACEUTICAL COMPANY AND THE EUROPEAN FOUNDATION FOR THE STUDY OF DIABETES AND JDRF. EACH OF US CONTRIBUTES SOMEWHERE BETWEEN A QUARTER OF A MILLION AND A HALF A MILLION EACH YEAR. IN THAT PARTICULAR INSTANCE, WE GOT THE MAJOR PHARMACEUTICAL COMPANY TO GIVE UP THEIR INTELLECTUAL PROPERTY POLICIES FOR THE PURPOSES OF THIS PARTICULAR GRANT BECAUSE WE SIMPLY SAID WE WERE PREPARED TO LET THE INVESTIGATORS DO IT, AND WE DIDN'T WANT TO CREATE ANY ADDITIONAL OBSTACLES. THEY DID AGREE TO DO THAT. SO THERE MAY BE ONE-OFF KIND OF NEGOTIATIONS THAT,

FOR THE SAKE OF EITHER PUBLIC RELATIONS VALUE OR WHATEVER PURPOSES, YOU CAN DO INDIVIDUAL THINGS.

NOW, I THOUGHT ONE EXAMPLE OF WHERE WE FUNDED FOR-PROFIT COMPANIES ACTUALLY WAS THE CONSEQUENCE OF OUR PERCEPTION THAT WE NEEDED NEW HUMAN EMBRYONIC STEM CELL LINES, AND WE ACTUALLY FUNDED A FEW COMPANIES OUTSIDE THE UNITED STATES AND ONE COMPANY ACTUALLY WITHIN THE UNITED STATES. AND IN THOSE CIRCUMSTANCES OUR MAIN INTEREST WAS TO MAKE NEW --

CHAIRMAN PENHOET: EXCUSE ME JUST A SECOND. DR. GOLDSTEIN, WE NOW HAVE YOUR SLIDES.

DR. GOLDSTEIN: GO TO SLIDE 6.

CHAIRMAN PENHOET: THANK YOU.

DR. BRYANT: IF YOU E-MAIL THEM TO US, WE CAN GET THEM RIGHT AWAY.

DR. GOLDSTEIN: I'LL JUST KEEP CHATTERING. IF YOU FIND IT OFFENSIVE, JUST TURN ME OFF.

THE POINT ABOUT SLIDE 6 WAS TO BRING TO YOUR ATTENTION STEM CELL RESEARCH. THERE'S VERY LITTLE OF STEM CELL RESEARCH THAT'S READY FOR CORPORATE DEVELOPMENT THIS WEEK. BUT CREATING NEW STEM CELL LINES WITHOUT ANIMAL CONTAMINATION AND MAKING FRESH LINES AVAILABLE IN A DIFFERENT MODEL WE THOUGHT WOULD BE HELPFUL. AS A CONSEQUENCE, WE FUNDED A FEW COMPANIES WHO SEEM TO BE A LITTLE AHEAD OF THE CURVE. AND WHAT WE WERE INTERESTED

IN WITH THOSE COMPANIES WAS NOT SO MUCH IP AS DISSEMINATION OF PRECIOUS LINES IN THE FIRST INSTANCE TO AS WIDELY A GROUP OF PEOPLE AS POSSIBLE. OBVIOUSLY THAT ONLY APPLIES OUTSIDE THE UNITED STATES BECAUSE OF THE WARF MATERIALS FOR STUFF COMING INTO THE UNITED STATES.

BUT, FOR EXAMPLE, WHAT WE ESSENTIALLY AS COMPANIES WE SAY YOU CAN'T CHARGE OUR JDRF-FUNDED RESEARCHERS YOUR CURRENT STANDARD RATE OF FIVE OR $6,000 BECAUSE THAT WON'T WORK. THEY SAID, OKAY, THAT'S FINE. WE ALSO SAID YOU HAVE TO GIVE IT OUT RATHER FREELY WITH VERY MODEST MTA'S, ETC., ETC., ETC. AND MOST PEOPLE AGREED TO DO THAT, I THINK, FOR TWO REASONS. ONE IS THEY THOUGHT THAT THEY WEREN'T GOING TO MAKE A LIVING SELLING STEM CELL LINES ANYWAY. AND THE IDEA WAS TO DISSEMINATE THEM WIDELY SO PEOPLE COULD ACTUALLY USE THEM FOR VALUABLE OTHER THINGS.

AND SO WE DIDN'T HAVE ANY PROBLEMS GETTING COMPANIES TO AGREE TO THOSE KIND OF WHAT I WOULD CALL OUR TYPICAL PRINCIPLES OF ACADEMIC FREEDOM AND DISSEMINATION OF INFORMATION.

THE NEXT SLIDE, NO. 7, RAISES THE QUESTION OF IF THIS RESEARCH IS SO EARLY, JUST WHAT IS IT THAT COMPANIES WILL BE ABLE TO BRING TO THE TABLE THIS WEEK ANYWAY GIVEN, AS I SAID, THE DIFFICULTIES WITH ETHICAL OVERSIGHT, THE DIFFICULTIES WITH THE PERCEPTION AND HOW

COMPANIES WANT TO PROTECT THEIR INTELLECTUAL PROPERTY, THAT PERHAPS DIFFERENT MOTIVATIONS, COMPANIES WANT TO MAKE MONEY, AND, THEREFORE, WE OCCASIONALLY, AND I'M NOT BEING SPECIFIC, WE OCCASIONALLY HEAR PROMISES THAT DON'T SEEM TO BE SCIENTIFICALLY DELIVERABLE LIKE WE'RE GOING TO BE IN CLINICAL TRIALS NEXT YEAR. SO WE ACTUALLY GIVE SOME THOUGHT TO THAT PERIODICALLY. SHOULD WE FUND NOT-PROFITS OR SHOULD WE FUND ONLY NOT-PROFITS? WHAT SHOULD WE DO WITH COMPANIES?

AND MORE INTERESTINGLY, WHEN WE HAVE A BIG PROGRAM COMPANY PROJECT-TYPE EVENT, OF WHICH THERE'S A COMPANY COMPONENT, HOW DO WE HANDLE THOSE COLLABORATIONS?

THE BOTTOM LINE FOR US IS IF THE PROPOSAL IS SCIENTIFICALLY APPROVED AND IT LOOKS LIKE IT'S GOING TO GET THE JOB DONE, WE'LL BEND OVER TO MAKE IT WORK.

NOW, THE LAST SLIDE, MERCIFULLY, IS SIMPLY ENTITLED -- IT'S THE ARTICLE FROM *NATURE BIOTECH*, WHICH YOU HAVE THE REFERENCE HERE, CALLED THE "GATEKEEPERS OF HUMAN EMBRYONIC STEM CELL PRODUCTS." AND ON FRIDAY IN *SCIENCE* THERE WILL BE AN ARTICLE CONCERNING THE IMPEDIMENT TO RESEARCH BECAUSE OF THE WARF PATENT.

MY STAFF WANTED YOU TO HEAR LATE BREAKING NEWS. THIS IS NOT ANYTHING NEW TO PEOPLE LIKE US WHO HAVE BEEN OUT THERE, BUT I DON'T KNOW IF IT BEGS THE QUESTION, OR THE QUESTIONS THAT WE WRESTLED WITH OVER TIME IS FUNDING

PEOPLE TO CREATE STEM CELL LINES, PARTICULARLY IN THE INTERNATIONAL COMMUNITY, AND PARTICULARLY TO HAVE THEM WIDELY DISSEMINATED UNDER REALLY MUCH EASIER TO DO RESEARCH AND CLINICAL APPLICATION AND ALSO TO OVERCOME THE, I WOULD SAY, WHAT PEOPLE IN THE UNITED STATES SEE AS THE CONTINUED IMPEDIMENT OF ACTUALLY USING A WARF LINE TO DISCUSS CLINICAL THERAPY.

AND SO AS FAR AS I KNOW, NOBODY HAS TAKEN THE PATENT TO TRY TO BREAK IT IN THE UNITED STATES, AND THAT'S NOT PART OF OUR DISCUSSION HERE. BUT I MENTION IT ONLY BECAUSE IT INDIRECTLY AFFECTED SOME OF OUR DECISIONS, PARTICULARLY IN THE CREATION OF CELL LINES AND PARTICULARLY IN HOW THEY WERE USED, SO WE WANTED TO SET UP A MODEL THAT WE THOUGHT WOULD BE APPROPRIATE ACADEMIC MODEL. AND FORTUNATELY MOST OF OUR PARTNERS LIKE, FOR EXAMPLE, IN THE UNITED KINGDOM, SWEDEN, SINGAPORE, ETC., ALL THOSE PEOPLE BELIEVED THAT WAS A GOOD THING TO DO ANYWAY. SO WE HAVE THIS KIND OF GOOD BEHAVIOR OUTSIDE THE UNITED STATES AND DIFFICULT BEHAVIOR WITHIN THE UNITED STATES, AND WE DON'T KNOW EXACTLY HOW THAT'S GOING TO WORK OUT.

AND LAST BUT NOT LEAST, AND THEN I'M SURE I WILL TRY TO ANSWER QUESTIONS THAT PEOPLE HAVE, LAST BUT NOT LEAST IS WHAT I WOULD CALL THE RESEARCH COLLABORATIONS ARE GOING TO BE OCCURRING PRESUMABLY WITH YOUR FUNDED

RESEARCH IN CALIFORNIA AND PEOPLE OUTSIDE CALIFORNIA IN A VARIETY OF WAYS. AND WHEN IT COMES TO HOW YOU NEGOTIATE INTELLECTUAL PROPERTY ON THOSE KIND OF ISSUES AND WHAT HAPPENS WHEN PEOPLE USE DERIVATIVE MATERIAL FROM ELSEWHERE AND HAVE THAT CROSS THE STATE BORDERS, THE FUN IS ONLY STARTING HERE IN TERMS OF THAT.

I JUST WANT TO BRING TO YOUR ATTENTION THAT THE PHOEBE BERMAN BIOETHICS INSTITUTE AT JOHNS HOPKINS TACKLED THE PROBLEM IN THE UNITED STATES, THE PROBLEMS OF INTERSTATE COLLABORATION IN STEM CELL RESEARCH. THEY SHOULD BE PUBLISHING SHORTLY A SUMMARY OF LAST DECEMBER'S MEETING.

BUT WHAT IT WILL SAY IS THAT THE VARIED RULES IN THE STATES PRESENT KIND OF A WET BLANKET FOR COLLABORATIONS BECAUSE PEOPLE JUST, YOU KNOW, ARE AFRAID TO WORK IN THESE UNCERTAIN ENVIRONMENTS. WHERE WE'VE DONE COLLABORATIONS OUTSIDE THE UNITED STATES WITH THOSE MEDICAL RESEARCH COUNCILS AND COMPANIES, AND PARTICULARLY LIKE IN PLACES LIKE SINGAPORE, WHICH HAS A VERY STRONG MODEL FOR FUNDING NOT-PROFITS AND FOR-PROFITS BY PUTTING ALL THE MONEY UNDER ONE HEAD. AND SINGAPORE HAS ACTUALLY THOUGHT A LOT ABOUT POLICY ISSUES LIKE THAT, BUT THEY HAVE BEEN ABLE TO PROMULGATE A POLICY THAT BOTH ENCOURAGES THE RESEARCH AND ENCOURAGES THE DISSEMINATION OF THE PRODUCTS OF RESEARCH, YET RETAINS SOME INTEREST SO

THAT THE FUNDERS MAINTAIN A KIND OF A POTENTIAL SHARE IN THE PROFITS.

AND THEN LAST BUT NOT LEAST, ONE OF THE PROBLEMS THAT WE PERIODICALLY FACE IS THAT ABOUT EVERY SIX MONTHS THERE'S AN ARTICLE THAT SAYS DR. X, WHO MADE A DISCOVERY TEN YEARS AGO, JUST SOLD THAT FOR $100 MILLION AND THE UNIVERSITY IS GETTING $200 MILLION. AND OUR BOARD MEMBERS SOMETIMES HAVE THE IMPRESSION THAT THAT'S AN EVERYDAY OCCURRENCE AND THAT WE SHOULD SET UP ALL THESE POLICIES TO CAPTURE THAT. WHEREAS, I THINK MOST OF US KNOW THAT'S NOT NECESSARILY AN EVERYDAY OCCURRENCE, AND YOUR ABILITY TO CAPTURE THE NEXT $100 MILLION GIFT FROM AN INTELLECTUAL DISCOVERY OCCURS SO SELDOM THAT YOU WONDER SOMETIMES ABOUT HOW MANY LAWYERS TO HIRE AND HOW MANY CONTRACTS TO EXECUTE TO MAKE THIS HAPPEN.

EVEN OUR LITTLE BITTY PROGRAM, I CAN TELL YOU, IT TAKES MONUMENTAL AMOUNTS OF LAWYER TIME, AND WE DON'T HAVE THE IN-HOUSE COUNSEL, BUT AT SOME POINT IN TIME SOMEBODY HAS GOT TO ASK THE QUESTION OF COST BENEFIT AND HOW THAT GOES.

SO LET ME CLOSE THERE. I DON'T KNOW IF I'VE ADDED TO YOUR THINKING OR NOT, BUT TRIED TO.

CHAIRMAN PENHOET: IT'S VERY HELPFUL. THANK YOU. CAN WE MAYBE RETURN TO THIS THORNY ISSUE OF SHARING THE PROFITS? YOU DID MENTION EARLY ON IN YOUR TALK THAT

YOU, QUOTE, UNQUOTE, GET A SHARE OF THE PROFITS SOMETIMES WHEN YOU FUND THE FOR-PROFIT. HOW IS THAT GENERALLY STRUCTURED? ARE THEY LOANS THAT NEED TO BE PAID BACK, OR IS IT ACTUALLY PROFITS OR IS IT ROYALTY? IN GENERAL, YOU ALSO SAID LATER THAT YOU NEGOTIATE EACH ONE OF THESE SEPARATELY, SO YOU MAY HAVE A MIX OF THESE THINGS. COULD YOU GIVE US SOME FLAVOR OF THE RETURN TO JDRF THAT'S EMBEDDED IN SOME OF YOUR CONTRACTS WITH THE FOR-PROFIT SECTOR?

DR. GOLDSTEIN: YES. IT'S TYPICALLY A PERCENTAGE OF PROFITS AT SOME FUTURE POINT. AND THE PERCENTAGES IN THEORY ARE SUPPOSED TO BE SET IN SOME FASHION IN RELATION TO THE DEGREE OF YOUR INVESTMENT. BUT I HAVE TO TELL YOU THAT WE DON'T HAVE A POLICING SYSTEM TO REALLY UNDERSTAND WHAT THE PERCENTAGE OF OUR INVESTMENT IS VERSUS WHAT THE PERCENTAGE OF THE COMPANY INVESTMENT IS. AND, IN FACT, WE HAVE THAT DIFFICULTY EVEN WITH UNIVERSITIES BECAUSE IF WE GIVE SOMEBODY A MILLION DOLLAR GRANT AND THEY'RE A SMALL BUSINESS, THEY'RE GOING SAY EVERY NICKEL THEY'VE EVER RAISED TO THAT POINT IN TIME IS PART OF THEIR CONTRIBUTION AND, THEREFORE, SIGNIFICANTLY DILUTES OUR PERCENTAGE.

WHAT WE'VE DONE REALLY, TO BE HONEST, IS RATHER THAN ARGUE THAT TILL WE'RE BLUE IN THE FACE, IS WE COME DOWN TO SOME NUMBER THAT BOTH SIDES CAN LIVE WITH, WHICH

IS USUALLY 2, 3, 4, 5, 6, 7 PERCENT RANGE, NOT 10, 20, 30 PERCENT RANGE AND WITH LOOSE GUIDELINES. WHAT IT REALLY DOES IS NOT WHAT SHOULD BE DONE IN A PERFECT WORLD. WHAT IT REALLY DOES IS SET UP A FUTURE NEGOTIATION WITH SOME GUIDING PRINCIPLES. AND WE HAVEN'T CALLED THE QUESTION THAT OFTEN, AND WE HAVEN'T PRODUCED ANY PROFITS YET, SO WE HAVEN'T BEEN CAUGHT WITH THAT. BUT IF IT WERE FIVE YEARS FROM NOW AND SOMEBODY REALLY DISCOVERED SOMETHING, PROBABLY THE KIND OF CONTRACTS WE'VE EXECUTED ARE TOO THIN TO BE ONEROUS AND HOLD UP IN TERMS OF LITIGATION.

ON THE OTHER HAND, WHAT WE'VE RELIED UPON TO SOME EXTENT IS WE ASSUME THAT THE RECIPIENTS OF FUNDING DO NOT WANT TO SEE A HEADLINE IN THE NEWSPAPER THAT THEY'VE SOMEHOW CHEATED THE FOUNDATION.

NOW, THE OTHER SOLUTION, WE'VE HAD SOME COMPANIES SAY, LOOK, LET'S NOT WORRY ABOUT ANY OF THIS. WE WILL PAY YOU, ASSUMING THIS IS PROFITABLE, WE WILL PAY YOU BACK THREE TIMES OR FOUR TIMES WHATEVER YOUR GRANT -- YOU KNOW, THE MILLION DOLLARS YOU GAVE US, WE'LL PAY YOU BACK FOUR OR FIVE MILLION AT SOME FUTURE DATE OUT OF PROFITS. I DON'T HAVE TO TELL YOU ABOUT HOW THE ACCOUNTING WORLD NEGOTIATES PROFITS. WE'VE ACCEPTED THAT KIND OF A FRAMEWORK IN ORDER TO GET GOING WITH THE WORK.

AGAIN, IF YOU REALLY WANT TO DO IT CORRECTLY, YOU HAVE TO PUT THAT STUFF INTO CONTRACT LANGUAGE AND BE

VERY SERIOUS ABOUT IT. SO WE'VE TAKEN A MIDDLE GROUND. WE PROBABLY WON'T CHANGE THAT IN ANY SIGNIFICANT WAY UNTIL WE SCALE UP TO BIGGER AMOUNTS.

CHAIRMAN PENHOET: OKAY. WE HAVE SOME QUESTIONS HERE IN SAN FRANCISCO. MICHAEL GOLDBERG.

MR. GOLDBERG: DR. GOLDSTEIN, HOW HAS JDRF DEALT WITH ISSUES RELATED TO AFFORDABILITY AND ACCESS?

DR. GOLDSTEIN: WE'VE INCLUDED, FOR EXAMPLE, IN ALL OF OUR -- EVERY GRANT THAT WE'VE MADE TO A COMPANY IN TERMS OF PRODUCING STEM CELL LINES, ETC., WE'VE INCLUDED A SENTENCE OR TWO ABOUT ONE OF THE REASONS WE WANT FREE DISSEMINATION AND ACCESS TO PRODUCTS THAT COME FROM THOSE STUDIES TO SUPPORT, YOU KNOW, JUSTICE FOR THE UNDERSERVED. BUT WE HAVE NOT CREATED LANGUAGE WITH TEETH TO ENFORCE THAT I THINK IN PART BECAUSE WE JUST DON'T SEE A THERAPY COMING NEXT WEEK, OR WE DON'T HAVE A THERAPEUTIC GRANT. WE HAVE STUDIES IN ANIMALS. THE MOST WE'VE GOT GOING ARE PEOPLE DOING MOUSE WORK AND CURING DIABETIC MODELS OF MICE WITH SOMETHING THAT WAS DERIVED FROM A STEM CELL LINE. AND WE HAVEN'T BEEN REALLY CONFRONTED WITH THE POSSIBILITY THAT WE'RE GOING TO HAVE A THERAPY.

ORGANIZATIONALLY WE INCLUDE LANGUAGE LIKE THAT. AND IN OUR STEM CELL OVERSIGHT COMMITTEE, WHICH WE HAVE A VERY ROBUST GROUP AND HAVE HAD FOR SEVERAL YEARS A KIND

OF REAL GOOD MODEL, THE ISSUE OF JUSTICE AND HOW THE POTENTIAL PRODUCTS OF RESEARCH ARE GOING TO BE UTILIZED IS ALWAYS DISCUSSED. AND PARTICULARLY IN OUR PARTNERSHIPS WITH PLACES LIKE SWEDEN AND THE UK, THESE ARE ALSO ISSUES THAT ARE ADDRESSED. BUT I HAVE TO TELL YOU THAT I DON'T SEE THAT THEY'RE ADDRESSED IN AN EXPLICIT, CONTRACTUAL MANNER THAT ACTUALLY GUARANTEES SOMETHING. I THINK THE ONLY GUARANTEE YOU HAVE TO MAKE THAT HAPPEN AT THE MOMENT IS IN THE COURT OF PUBLIC OPINION AND PERSUASIVENESS.

CHAIRMAN PENHOET: OTHER QUESTIONS FROM THE MEMBERS OF THE TASK FORCE IN SAN FRANCISCO?

DR. PRIETO: I HAVE ONE HERE IN ELK GROVE. DR. GOLDSTEIN, THIS IS FRANCISCO PRIETO. I'M THE TYPE 1 DIABETES ADVOCATE ON THE ICOC. I WAS INTRIGUED BY THE COMMENT YOU MADE ABOUT SOME OF YOUR EUROPEAN PARTNERS, WHICH HAVE, OF COURSE, NATIONAL HEALTH SYSTEMS. SO IF THE GOVERNMENT OF SWEDEN, FOR EXAMPLE, HAS A SUBSTANTIAL INVESTMENT IN SOMETHING THAT COULD LEAD TO A THERAPY, DO THEY NOT HAVE LANGUAGE IN PLACE NOW GUARANTEEING SOME SORT OF PRICING FOR THEIR PURCHASE OF THAT THERAPY?

DR. GOLDSTEIN: TO MY KNOWLEDGE THEY DO NOT HAVE LANGUAGE, BUT THEY HAVE PUBLICLY STATED PHILOSOPHY. AND THAT WOULD BE A VERY POWERFUL TOOL. I DON'T THINK THEY'VE GOTTEN AROUND TO WRITING LANGUAGE, BUT THEY

ALWAYS HAVE IN ALL THOSE AGREEMENTS, YOU KNOW, THE PUBLIC MODEL. YOU HAVE TO FREELY DISSEMINATE THE RESOURCE AND THAT SORT OF THING.

DR. PRIETO: I HAVE ONE OTHER QUESTION ABOUT THE LIABILITY AND INDEMNIFICATION ISSUES THAT YOU MENTIONED EARLIER. OF COURSE, COMMERCIAL COMPANIES, PHARMACEUTICAL COMPANIES, ARE DEALING WITH THIS ALL THE TIME IN CLINICAL TRIALS. AND I JUST WONDER HOW ARE THOSE PRIVATE COMPANIES DEALING WITH IT NOW? I HAD ALWAYS JUST ASSUMED THAT THEY WERE INSURED.

DR. GOLDSTEIN: THAT'S WHAT WE ASSUME, BUT WE TRY TO BE VERY EXPLICIT ABOUT THAT. IN CLINICAL TRIALS WE'RE EXPLICIT ENOUGH TO REQUIRE INDEMNIFICATION AND HOLD HARMLESS CLAUSES. AND WHERE PEOPLE ARE ACTING ON BEHALF OF AN INSTITUTION THAT DOESN'T LOOK LIKE IT HAS DEEP POCKETS, WE DEMAND INSURANCE COVERAGE OF SOME AMOUNT SO THAT WE'RE OFF THE HOOK EITHER WAY.

DR. PRIETO: YOU INCLUDE THAT IN GRANT LANGUAGE?

DR. GOLDSTEIN: THAT IS CORRECT, PARTICULARLY FOR CLINICAL TRIALS. WHEN IT'S NOT INVOLVING HUMAN SUBJECTS, IT'S OF LESS INTEREST TO US.

CHAIRMAN PENHOET: ANY OTHER QUESTIONS FROM OUR OTHER SITES? IF NOT, DO WE HAVE QUESTIONS FROM THE AUDIENCE HERE IN SAN FRANCISCO?

MR. SIMPSON: JOHN SIMPSON FROM THE FOUNDATION

FOR TAXPAYER AND CONSUMER RIGHTS. DR. GOLDSTEIN, WAS IT THE CASE THAT YOU SAID THAT YOU BELIEVE THAT THE WARF PATENTS ARE THWARTING STEM CELL RESEARCH IN THE UNITED STATES, BLOCKING RESEARCH? DID I UNDERSTAND THAT CORRECTLY?

DR. GOLDSTEIN: WHAT I WAS SAYING IS THAT I'VE GIVEN YOU A REFERENCE IN THE LAST SLIDE TO AN ARTICLE IN *NATURE BIOTECH* THAT SAYS THAT, AND *SCIENCE* MAGAZINE HAS A PIECE ON FRIDAY OF THIS COMING WEEK THAT SAYS EXACTLY THAT. I WOULD SAY DEFINITELY OUR BELIEF IS THAT THE WARF PATENTS HAVE BEEN A MAJOR INHIBITION TO PRODUCTIVE SCIENTIFIC RESEARCH THAT'S POTENTIALLY AVAILABLE BECAUSE IT'S HAD A DAMPENING EFFECT ON DISSEMINATION OF STEM CELL LINES. AND THE NIH HASN'T NECESSARILY FIXED THAT PROBLEM, AND THE ORIGINAL STEM CELL LINES ARE INADEQUATE FOR ALL THE RESEARCH THAT WE THINK NEEDS TO BE DONE ANYWAY.

MR. SIMPSON: YOU'RE NOT JUST CITING THE ARTICLE. YOU'RE ASSERTING THAT THAT'S YOUR BELIEF AS WELL, CORRECT?

DR. GOLDSTEIN: WELL, THAT'S OUR ASSUMPTION BECAUSE OUTSIDE THE UNITED STATES, IT'S A FREE PROCESS. SO JUST BY DEFAULT WE'VE CONCENTRATED EFFORTS OUTSIDE THE UNITED STATES TO CREATE NEW AND BETTER STEM CELL LINES, AND THAT PROCESS HAS OCCURRED AND IS CURRENTLY

FLOURISHING. AND PRESUMABLY THE FRUITS OF RESEARCH FROM THOSE BETTER AND MORE IMPROVED LINES WILL COME SOONER AND BE MORE ROBUST THAN STICKING WITH THE ORIGINAL NIH LINES.

MR. SIMPSON: THANK YOU.

CHAIRMAN PENHOET: THANK YOU. ANY OTHER COMMENTS?

MR. MARGUS: THIS IS BRAD MARGUS. I'M CEO OF PERLEGEN SCIENCES. YOU MENTION THAT YOU FEEL COMPELLED TO NEGOTIATE CASE BY CASE DIFFERENT AGREEMENTS WITH DIFFERENT COMPANIES. WHAT IS YOUR OPINION AS FAR AS IF CIRM HAD A SINGLE MORE RIGID POLICY? HOW MUCH WOULD IMPEDE YOU?

DR. GOLDSTEIN: WE STARTED OUT BY SAYING WE'D LIKE TO CREATE, YOU KNOW, ONE CONTRACT THAT FITS ALL SIZES. IT JUST DIDN'T WORK. SO JUST A PRACTICAL COMMENT IS THAT ONE SIZE DOESN'T FIT ALL BECAUSE IN OUR CASE WE HAVE FUNDING THAT VARIES FROM SMALL AMOUNTS TO LARGER AMOUNTS, AND THERE ARE DIFFERENT REQUIREMENTS, DIFFERENT DESIRES. AND WE'VE HAD A HECK OF A TIME TRYING TO HAVE ONE CONTRACTUAL BLANK APPROACH FIT ALL SIZES.

SO IN A PERFECT WORLD, I WOULD LOVE TO HAVE ONE PIECE OF PAPER THAT EVERYBODY SIGNED UP ON AND IT FIT. IT JUST HASN'T WORKED OUT EXACTLY THAT WAY.

MR. REED: I WAS CURIOUS. WHAT RETURNS -- YOU GET MATCHING GRANTS FROM THE NIH. I HAVE A REAL CONCERN

WITH BEING LOCKED INTO ANY ONE TYPE OF CONTRACT WHICH MIGHT CONFLICT WITH BAYH-DOLE AND IN ANY WAY INFRINGE ON THE ABILITY TO GET MORE MONEY BACK FROM THE NIH AT A TIME WHEN WE HAVE A MORE FRIENDLY ADMINISTRATION IN THAT AREA. WHAT'S YOUR THOUGHTS?

DR. GOLDSTEIN: I'M NOT SURE I UNDERSTOOD YOUR POINT. ARE WE TALKING ABOUT NOT-PROFITS OR FOR-PROFITS?

MR. REED: I'M NOT SURE. I JUST KNOW THAT, LIKE, THE ROMAN REED ACT IS THE ONLY THING I REALLY KNOW ABOUT IN-DEPTH, AND THAT'S WE SPENT IN CALIFORNIA $6 MILLION IN TAX MONEY, BUT WE GOT BACK $26 MILLION IN MATCHING GRANTS FROM NIH AND OTHER SOURCES. SO IN OTHER WORDS, THE SEED MONEY THAT WE HAD WE GOT BACK A MUCH GREATER RETURN FROM THE NIH. AND I KNOW THAT TO GET MONEY BACK FROM THE NIH, YOU HAVE TO NOT CONFLICT WITH THE BAYH-DOLE PROPERTY RIGHTS AND STUFF LIKE THIS. SO I JUST --

DR. GOLDSTEIN: MOST OF THE FOR-PROFITS THAT YOU'RE TALKING ABOUT ARE NOT GOING TO HAVE A LOT OF NIH GRANTS PER SE. SO I DON'T SEE HOW THEY'RE GOING TO LEVERAGE THAT. SO EVEN THE SMALL BUSINESS INNOVATIVE RESEARCH PROGRAM, WHICH IS ADMINISTERED DIFFERENTLY AT DIFFERENT INSTITUTES. FOR INSTANCE, THE DIABETES INSTITUTE WILL GIVE YOU HALF A MILLION DOLLARS AND COMBINE A PART I AND PART II APPLICATION. SOMEBODY EARLY

ON IN THE TALK HAD GIVEN OUT WHAT ARE THE ORIGINAL RULES OF THE SBIR PROGRAM. NIH WILL GIVE YOU A COUPLE MILLION DOLLARS, SO PEOPLE HAVE VIEWED THAT IN A MORE SERIOUS WAY.

THERE MAY BE SOME OF THE LARGER VACCINE PROGRAMS THAT COMPANIES ARE GETTING MONEY FROM NIH, BUT NOT IN THIS PARTICULAR WORLD OF RESEARCH. I DOUBT THAT THERE'S VERY MUCH MONEY FROM NIH ON THE STREET FOR COMPANIES IN THE GRAND SCHEME OF THINGS IN THIS WORLD. SO I DON'T KNOW HOW YOU LEVERAGE THAT TO GET BACK MONEY.

IN TERMS OF THE NOT-PROFITS, WERE PUTTING HARDLY ANY IMPEDIMENTS IN THE WAY AND SIMPLY SAYING WE'LL STAND BY AND TAKE SOME AMOUNT OF THE ULTIMATE PROFITS IN THE FORM OF ROYALTIES OR WHATEVER, AND WE'LL LET YOU REINVEST IT IN YOUR RESEARCH PROJECT. AND IF WE EVER GET ANY MONEY, WE'LL REINVEST IT IN RESEARCH.

IN THE FOR-PROFIT WORLD, WE'RE HAVING, INSTEAD OF A DISCUSSION, WE'RE WRITING UP SOMETHING LIKE A CONTRACT TO ENFORCE THEM.

CHAIRMAN PENHOET: OKAY. IF THERE ARE NO OTHER COMMENTS, WE'LL THANK DR. GOLDSTEIN FOR A VERY INFORMATIVE PRESENTATION. WE REALLY APPRECIATE YOUR PARTICIPATION TODAY. AND WE'LL MOVE ALONG TO OUR NEXT SPEAKER, WHO IS STEPHEN JUELSGAARD.

DR. GOLDSTEIN: THANK YOU VERY MUCH.

(APPLAUSE.)

CHAIRMAN PENHOET: MARY MAXON INFORMS ME THAT DR. GOLDSTEIN'S SLIDES HAVE BEEN SENT TO THE REMOTE SITES, AND NOW DR. JUELSGAARD'S HAVE AS WELL. HOPEFULLY YOU'VE RECEIVED THOSE THERE.

DR. JUELSGAARD IS THE EXECUTIVE VICE PRESIDENT, GENERAL COUNSEL, SECRETARY, AND CHIEF COMPLIANCE OFFICER OF GENENTECH. WE'RE VERY PLEASED TO HAVE YOU JOIN US THIS MORNING. THANK YOU, STEPHEN.

DR. JUELSGAARD: THANK YOU, ED. AND THANK YOU FOR THIS OPPORTUNITY TO TALK A LITTLE BIT FROM A DIFFERENT POINT OF VIEW THAN THE FIRST TWO THAT YOU HAVE HEARD FROM. I REPRESENT A COMPANY THAT'S INVOLVED IN MAKING AND SELLING MEDICAL PRODUCTS, IN THIS CASE BIOTECHNOLOGY-DERIVED PHARMACEUTICAL PRODUCTS, SO I'M APPROACHING THINGS FROM A DIFFERENT END OF THE SPECTRUM.

BEFORE I REALLY GET INTO THE PRESENTATION, SORT OF AS I'VE HEARD THE INITIAL SPEAKERS, THREE COMMENTS THAT I'D LIKE TO MAKE. FIRST OF ALL, I AM A LAWYER AND PART OF MY JOB I SEE AT THE END OF THE DAY IS TO SORT TO SWIM AGAINST THE STREAM OF SOME OF THE REFERENCES TO LAWYERS THAT I'VE HEARD MADE THIS MORNING. WE REALLY TRY TO BE PRODUCTIVE AT THE END OF THE DAY.

DR. PIZZO: IT'S A LIFETIME JOB.

DR. JUELSGAARD: YOU'RE RIGHT, PHIL. THE SECOND

IS THAT, AS I'VE LISTENED TO, IN PARTICULAR, GREG MILMAN TALK IN THE FIRST PRESENTATION, I THINK IT'S REALLY IMPORTANT AT THE END OF THE DAY FOR THE CIRM TO IDENTIFY WHAT ITS OBJECTIVES AND PRIORITIES ARE AND MATCH THE PROGRAMS TO THEM. AS GREG TALKED ABOUT THE PRIORITIES OF HIS PROGRAMS, REALLY RELATE TO ECONOMIC DEVELOPMENT. AND WHEN YOU INVOLVE SOMETHING LIKE THAT, YOU DEVELOP A DIFFERENT LIST OF CRITERIA THAN YOU MIGHT FOR SCIENTIFIC ADVANCEMENT. AND AT THE END OF THE DAY, IF YOU HAVE TOO MANY PRIORITIES, YOU WILL FIND THAT THEY CONFLICT AND YOU WON'T BE ABLE TO ACHIEVE YOUR OBJECTIVE. SO I THINK IT'S REALLY IMPORTANT TO LINE UP WHAT YOUR PRIORITIES ARE AND PUT THE PROGRAM TOGETHER TO ACHIEVE THOSE AND NOT TO HAVE TOO MANY CONFLICTING ONES.

AND THE THIRD IS, AND ACTUALLY THIS POINT WAS MADE IN PART OF THE LAST DISCUSSION, AND THAT IS IT'S VERY HARD TO SEE, PARTICULARLY IN THE ENVIRONMENT THAT I DEAL IN, THAT THERE CAN EVER BE A ONE SIZE FITS ALL MODEL, THAT IT IS A VERY COMPLICATED WORLD, AND THERE ARE A LOT OF DIFFERENT NEEDS. AND I DO THINK IF YOU ARE GOING TO GET INVOLVED, IN PARTICULAR, WITH THE PRIVATE SECTOR, THAT NOTION THAT YOU CAN HAVE A SINGLE MODEL WHICH WILL SERVE ALL ENDS OF THE SPECTRUM IS A VERY, VERY DIFFICULT ONE. I DO THINK THERE WILL HAVE TO BE SOME FLEXIBILITY BUILT IN TO DEAL WITH THE PRIVATE SECTOR

WORLD SHOULD YOU CHOOSE TO DO THAT.

TO SOME EXTENT MY PRESENTATION IS DIVIDED INTO TWO PARTS. THE FIRST PART REALLY TRIES TO ADDRESS A COUPLE OF THEMES WITH RESPECT TO THE INDUSTRY THAT I'M IN AND THE ROLE THAT WE PLAY AND SOME OF THE OBSTACLES THAT WE FACE TO GIVE YOU A BETTER SENSE, A BETTER GROUNDING ABOUT THAT. AND THEN THE SECOND IS TO DIVE IN A BIT INTO SOME OF THE ISSUES THAT WE SEE OR I SEE IN ANY EVENT IN WORKING WITH GOVERNMENT ORGANIZATIONS AND INTERACTING WITH THEM IN PARTICULAR WHERE FUNDING IS INVOLVED.

LET ME JUST SAY IN THAT REGARD THAT I DO WORK FOR GENENTECH. I'M AN OFFICER OF GENENTECH, BUT I'M NOT COMING HERE REPRESENTING GENENTECH, AS YOU WILL SEE IN ONE OF MY SLIDES. WE REALLY HAVE NOTHING TO DO WITH THE STEM CELL AREA SAVE FOR ONE EXCEPTION, WHICH I'LL POINT OUT IN A MOMENT. SO THESE VIEWS REALLY REFLECT, THE ONES I'M GOING TO PRESENT, ARE MY VIEWS ALTHOUGH OBVIOUSLY THEY HAVE BEEN DEVELOPED OVER A NUMBER OF YEARS WORKING AT GENENTECH. I'VE BEEN THERE NOW FOR 20 AND A HALF YEARS, SO THEY'RE BASED ON MY EXPERIENCE IN THE INDUSTRY AND IN PARTICULAR AT GENENTECH, BUT I WANT TO BE CLEAR THAT THESE ARE NOT GENENTECH'S VIEWS.

LET ME GIVE YOU A LITTLE BACKGROUND VERY QUICKLY ABOUT GENENTECH BECAUSE I WANT TO LEAD TO THE NEXT PART OF THIS. SO WE'RE THE OLDEST BIOTECHNOLOGY COMPANY

ESSENTIALLY IN THE WORLD FOUNDED IN 1976. WE'RE HEADQUARTERED JUST DOWN THE ROAD HERE IN SOUTH SAN FRANCISCO. WE HAD REVENUES LAST YEAR OF $6.6 BILLION, WE HAVE 12 MARKETED PRODUCTS TO TREAT THE DISEASES THAT ARE LISTED HERE. OUR BIGGEST PRODUCTS ARE IN THE CANCER AREA, BUT WE HAVE PRODUCTS TREATING HEART ATTACK AND STROKE, SEVERE ASTHMA, CYSTIC FIBROSIS, GROWTH HORMONE DEFICIENCY. WE HAVE 9800 EMPLOYEES, OF WHICH 8500 ARE LOCATED HERE IN CALIFORNIA. TO JUST REPEAT, WE'RE NOT INVOLVED AT ALL IN THE STEM CELL RESEARCH AREA. IT IS NOT SOMETHING THAT WE HAVE PUT IN OUR LINE OF SIGHT WITH ONE EXCEPTION, AND THAT'S THE AREA OF CANCER STEM CELLS, WHICH IS STARTING TO EVOLVE NOW.

AND THE NOTION THAT AT THE BASE ROOT OF SOME CANCERS, IF NOT ALL OF THEM, ARE A GROUP OF CELLS CALLED CANCER STEM CELLS WHICH GIVE RISE PERPETUALLY TO NEW CANCER CELLS. AND THERE THE OBJECT, IF THIS PROVES TO BE TRUE, AND A MECHANISM TO TREAT CANCER WILL BE TO DESTROY THESE CELLS RATHER THAN TO REGENERATE OR PERPETUATE THEM. SO A VERY DIFFERENT CONCEPT THAN THIS GROUP HAS IDENTIFIED AS A WAY FORWARD FOR NEW THERAPIES.

CHAIRMAN PENHOET: GENENTECH'S R&D BUDGET THIS YEAR WILL BE HOW MUCH MONEY?

DR. JUELSGAARD: $1.5 BILLION IN RESEARCH AND DEVELOPMENT, AND THAT'S ROUGHLY SPLIT. SO I'M GOING TO

TALK ABOUT THIS ACTUALLY IN THE NEXT SEVERAL SLIDES, RESEARCH AND DEVELOPMENT, BECAUSE THEY ARE TWO COMBINATIONS OF EFFORTS THAT AT THE END OF THE DAY ARE ESSENTIAL TO LEAD TO NEW THERAPIES. AND I THINK WHAT THE HOPE IS IS THAT WHAT COMES OUT OF ALL THESE EFFORTS ARE NEW THERAPIES, SO I THINK WE NEED TO UNDERSTAND THE SPECTRUM OF WHAT GOES ON IN DEVELOPMENT OF NEW THERAPIES.

WE SPEND ROUGHLY FOR EVERY ONE DOLLAR IN RESEARCH, WHICH I'LL TALK ABOUT A LITTLE MORE DETAIL, BUT WHICH IS REALLY TRYING TO IDENTIFY POTENTIAL PRODUCTS. FOR EVERY DOLLAR WE SPEND IN RESEARCH, WE SPEND $5 IN DEVELOPMENT. THAT'S ROUGHLY THE RATIO AT GENENTECH. IT'S VARIED OVER TIME BETWEEN ONE TO FOUR TO ONE TO FIVE, SO THAT'S ROUGHLY THE BREAKDOWN. THE LION'S SHARE OF MONEY REALLY GETS SPENT ONCE YOU'VE IDENTIFIED THAT LEAD POTENTIAL PRODUCT OF TRYING TO BRING IT TO THE MARKETPLACE.

SO I WANTED TO AGAIN BRIEFLY ADDRESS TWO AREAS, THE AREA OF RESEARCH, WHICH ESSENTIALLY IN THE CASE OF A COMPANY LIKE OURS, AND ED PRESENTED THIS SLIDE, IT'S THE TRANSLATION OF BASIC RESEARCH INTO APPLIED RESEARCH, ESSENTIALLY TAKING THINGS THAT HAVE ALREADY BEEN DEVELOPED AT A FUNDAMENTAL LEVEL, OFTEN DONE IN ACADEMIC INSTITUTIONS, ALTHOUGH, PHIL, WE DO DO SOME BASIC RESEARCH AT GENENTECH AS WELL.

DR. PIZZO: OUTSTANDING BASIC RESEARCH.

DR. JUELSGAARD: WE HAVE SOME EX-STANFORD FOLKS TO PROVE IT AS WELL.

DR. PIZZO: THEY DO THE MOST OUTSTANDING WORK.

DR. JUELSGAARD: WE'RE VERY PROUD TO HAVE THEM. AND ALSO FOR ZACH'S BENEFIT, SOME GREAT PEOPLE FROM UCSF.

DR. PIZZO: LITTLE LESS.

DR. JUELSGAARD: WE'LL MOVE ON. AND THE OTHER IS THE DEVELOPMENT OF NEW THERAPIES WHICH ESSENTIALLY, AS I INDICATED, TAKE SOMETHING THAT WE'VE IDENTIFIED MAY BE A POTENTIAL PRODUCT AND REALLY TRIES TO MOVE IT THROUGH ALL OF THE STEPS THAT ARE NEEDED TO DETERMINE AT THE END OF THE DAY WHETHER IT WILL BE A PRODUCT. AND ESSENTIALLY THAT MEANS PROVING OR DEMONSTRATING SAFETY AND EFFECTIVENESS. THOSE ARE THE TWO HALLMARKS OF A PRODUCT -- MUST BE TRUE OF A PRODUCT IN ORDER FOR IT TO BE SOLD CERTAINLY IN THE UNITED STATES.

SO MY FIRST SET OF POINTS THAT I WANT TO MAKE IS THAT INNOVATION, SORT OF THE KEY TO OUR INDUSTRY IS INNOVATION. AND A TREMENDOUS AMOUNT OF INNOVATION GOES ON IN THE PRIVATE SECTOR. FOR US, ONE OF THE PRIMARY MEASURES OF INNOVATION ARE PATENTS. AT THE END OF THE DAY, PATENTS ARE ALL ABOUT INVENTIONS THAT ARE MADE, NEW AND NOVEL IDEAS THAT HAVE BEEN REDUCED TO PRACTICE.

AND SO THE POINT I WANT TO MAKE IS WHILE THERE'S

OBVIOUSLY A LOT OF ATTENTION THAT'S PAID TO ALL OF THE RESEARCH THAT GOES ON IN ACADEMIC INSTITUTIONS, I THINK IT'S IMPORTANT TO NOTE THAT THERE'S A SIGNIFICANT AMOUNT OF INNOVATION AND RESEARCH THAT GOES ON IN COMPANIES. AND THE SLIDES THAT I'M GOING TO SHOW YOU, THESE ARE NOT AT THE END OF THE DAY DESIGNED ESSENTIALLY TO MAKE SOMETHING OUT OF GENENTECH. WHEN I PUT THIS PRESENTATION TOGETHER, I SORT OF DID IT IN THE LAST WEEK OR SO AFTER TALKING TO MARY ABOUT THIS BECAUSE I CAME TO THIS MORE RECENTLY TO MAKE THIS PRESENTATION. SO I PULLED EXISTING SLIDES THAT WE HAD AT GENENTECH AND DIDN'T TRY TO REMODEL THEM, SO YOU WILL SEE THAT SOME OF THESE SLIDES ILLUSTRATE US VIS-A-VIS OTHER COMPANIES ONLY FOR INTERNAL PURPOSES WHEN WE GENERATED THESE SLIDES. SO IGNORE THE PLACEMENT OF GENENTECH ON THESE, BUT I WANT TO USE THESE FOR A MORE FUNDAMENTAL POINT. THAT IS, IN THIS PARTICULAR CASE, THE AMOUNT OF INNOVATION THAT GOES ON WITHIN COMPANIES.

IN THE CASE OF GENENTECH, WE HAVE OVER 5600 GRANTED PATENTS BETWEEN THE UNITED STATES AND THE REST OF THE WORLD AND 5300 PENDING PATENT APPLICATIONS. NOT ALL OF THOSE WILL GIVE RIGHTS TO PATENTS, BUT WE HAVE DONE A TREMENDOUS AMOUNT OF RESEARCH WORK OVER THE YEARS AT GENENTECH, AND THIS GOES ON IN ALL OF THE COMPANIES THAT ARE OUT THERE. THIS IS REALLY A MAINSTAY OF WHAT THEY DO

AND WHAT WE DO.

AND SO AT THE ROOT OF THE DEVELOPMENT OF NEW THERAPIES IS A SIGNIFICANT AMOUNT OF INNOVATION THAT GOES ON INSIDE COMPANIES. JUST TO SORT OF REINFORCE THAT POINT, THIS IS SOMETHING THAT, AGAIN, WE'VE LOOKED AT, IN PARTICULAR IN THE AREA THAT WE'RE INVOLVED IN AND THE NUMBER OF PATENTS THAT HAVE BEEN GRANTED ESSENTIALLY TO VARIOUS COMPANIES OR INSTITUTIONS. IN OUR CASE WE'RE REALLY INVOLVED IN THE PEPTIDE OR PROTEIN AREA. THIS DATA WAS PULLED FROM THE U.S. PATENT AND TRADEMARK OFFICE DATABASE, AND THE ONE ON THE LEFT THEY HAVE A PARTICULAR CLASSIFICATION THAT RELATES TO PEPTIDE AND PROTEIN PATENTS. AND SO, AGAIN, MY POINT IS NOT TO IDENTIFY ANY PARTICULAR ORGANIZATION AND WHAT THEY DO, BUT ONLY TO MAKE THE POINT THAT A SIGNIFICANT AMOUNT OF RESEARCH INTO NEW THERAPIES IS GOING ON IN THE PRIVATE SECTOR.

AND AT THE END OF THE DAY, IF THE GOAL IS TO REALLY TRY AND BRING NEW THERAPIES FORWARD, THE PRIVATE SECTOR IS GOING TO BE A GREAT PLACE TO REALLY TURN TO TO POTENTIALLY HELP SUPPORT IN THAT REGARD, AS WELL AS OBVIOUSLY THE ACADEMIC AND OTHER NOT-FOR-PROFIT INSTITUTIONS.

SO I SORT OF MADE MY POINT, WHICH IS WHY IS THIS IMPORTANT OR RELEVANT? AND THAT IS BECAUSE THE AMOUNT OF FUNDAMENTAL TRANSLATIONAL RESEARCH, FOR THE MOST PART,

ALTHOUGH SOME OF IT'S BASIC, THAT GETS DONE IN THESE ORGANIZATIONS. AND SO IF ONE BELIEVES IT'S IMPORTANT TO PROVIDE THE RIGHT CONDITIONS WHICH WILL SUPPORT AND ENCOURAGE THAT RESEARCH -- I'LL COME BACK TO THAT LATER BECAUSE THERE ARE THINGS THAT IN MY VIEW YOU CAN DO WHICH WILL IMPEDE THAT PROPOSITION.

CHAIRMAN PENHOET: FOR THOSE OF YOU WHO ARE FOLLOWING THE PRESENTATION, STEVE IS NOW ON SLIDE 7.

DR. JUELSGAARD: I FORGOT ABOUT THAT. I'LL TRY TO, AS I ADVANCE THE SLIDES, IDENTIFY THE SLIDE THAT I'M ON.

SO THE NEXT THING THAT I WANTED TO TALK ABOUT IS DRUG DEVELOPMENT, WHICH IS, AS I SAID, WHERE ACTUALLY THE LION'S SHARE OF THE MONEY GETS SPENT, AND TO REALLY TALK ABOUT THREE THINGS BECAUSE I THINK IT'S IMPORTANT TO UNDERSTAND THIS FROM AN INDUSTRY PERSPECTIVE IN THAT DRUG DEVELOPMENT IS RISKY, LENGTHY, AND EXPENSIVE. AND SO TO TALK ABOUT THIS, YOU TALK ABOUT A STUDY THAT WAS DONE, FIRST OF ALL. THIS IS A STUDY PUBLISHED IN THE *JOURNAL OF HEALTH ECONOMICS* IN 2002. I'M GOING TO TALK A BIT ABOUT SOME MORE RECENT WORK THAT WE'VE DONE AT GENENTECH USING THIS SAME GROUP. A GROUP OF INDIVIDUALS, JOE DI MASI FROM TUFTS UNIVERSITY, RON HANSON FROM UNIVERSITY OF ROCHESTER, HENRY GROBOWSKI FROM DUKE UNIVERSITY, ALL AFFILIATED IN THE ECONOMICS AREAS OF THEIR ORGANIZATIONS,

PUT TOGETHER A STUDY TO LOOK AT THE COST OF RESEARCH AND DRUG DEVELOPMENT AROUND PARTICULAR PRODUCTS AND PUBLISHED THIS IN 1982.

AND THE TAKE-HOME IS IN THAT PERIOD OF TIME -- I'M SORRY -- 1992, AT THAT TIME THAT THE ACTUAL COST PER APPROVAL FOR A MOLECULE WAS $403 MILLION AND THAT THE CAPITALIZED COST, WHICH, IN ESSENCE, TAKES INTO CONSIDERATION THE COST OF CAPITAL, WHICH FOR US IS REALLY THE MORE APPROPRIATE WAY TO LOOK AT IT AS OPPOSED TO THE PURE OUT-OF-POCKET DOLLARS, BUT THE INVESTMENT COST ASSOCIATED WITH THE DEVELOPMENT OF PRODUCTS IS $802 MILLION PER PRODUCT. SO ROUGHLY THE COST TO BRING A NEW PRODUCT FORWARD. PARTICULARLY THESE ACCOUNTED FOR ALL COSTS. THESE WERE FAILURES AS WELL AS SUCCESSES BECAUSE, AS YOU WILL SEE, THERE ARE A FAIR NUMBER OF FAILURES THAT GO ON. SO WE THINK IT'S IMPORTANT TO CONSIDER IT ALL IN COST.

SO WE ASKED THIS GROUP LAST YEAR TO COME TO GENENTECH AND LOOK AT -- TO UPDATE THEIR DATA AND LOOK AT HOW WE DO THINGS AND WHAT WE DO AND WHERE OUR STRUCTURE FITS VIS-A-VIS THEIR ORIGINAL FINDINGS. AND THE NEXT SLIDES I'M GOING TO SHOW YOU RELATE TO THAT. AGAIN, I DON'T WANT TO FOCUS ON GENENTECH. THAT'S NOT THE PURPOSE OF THIS. I WANT TO MORE FOCUS ON THE DATA GENERICALLY AROUND THE ORGANIZATIONS INVOLVED.

SO THE FIRST IS WHAT WE CALL THE PROBABILITY OF TECHNICAL SUCCESS FOR COMPOUNDS ENTERING CLINICAL TESTING. WHAT IT ESSENTIALLY MEANS IS WHAT ARE THE CHANCES THAT YOU WILL BE SUCCESSFUL? WHAT ARE THE ODDS AT THE END OF THE DAY THAT WHEN YOU START CLINICAL TESTING, WHAT WILL COME OUT AT THE OTHER END AS A SUCCESSFUL PRODUCT? WHAT I DEMONSTRATE, I WANT TO MOSTLY FOCUS ON PHARMA AND BIOPHARMA, BIOPHARMA BEING ESSENTIALLY THE PART OF THE INDUSTRY WE'RE IN, THE BIOTECH AREA, THAT THE ODDS OF BEING SUCCESSFUL ARE SOMEWHERE AROUND ONE IN FIVE TO A LITTLE LESS THAN ONE IN THREE. SO TO BE CLEAR, MOST COMPOUNDS THAT WE START CLINICAL TESTING WITH, REMEMBER AT THIS POINT WE'VE IDENTIFIED THEM IN RESEARCH AS POTENTIALLY PROMISING CANDIDATES, WE'VE ALREADY TESTED THEM IN ANIMALS TO MAKE SURE WE'VE IDENTIFIED ANY POTENTIAL TOXICITY PROBLEMS BEFORE WE GET INTO MAN, ETC., BUT ONCE WE START IN HUMANS, THE ODDS OF SOMETHING SUCCESSFUL COMING OUT AT THE OTHER END ARE IN THIS RANGE.

SO MORE THINGS ARE GOING TO FAIL THAN ARE GOING TO SUCCEED. THAT'S JUST A GIVEN IN TERMS OF THE WAY THIS PROPOSITION WORKS. SO THIS IS A VERY RISKY ENTERPRISE, DEVELOPING PRODUCTS.

THE SECOND IS THE TIMELINES THAT ARE INVOLVED. I SAID THERE WERE THREE ISSUES HERE. THE OTHER IS THE

LENGTH. THESE ARE THE NUMBER OF MONTHS THAT ONCE YOU START CLINICAL DEVELOPMENT IT'S GOING TO TAKE BEFORE YOU GET SOMETHING OUT ON THE OTHER END IN TERMS OF A PRODUCT BROKEN INTO PHASE I TRIALS, PHASE II TRIALS, PHASE III CLINICAL TRIALS, AND THEN REGULATORY REVIEW OR TIME SPENT BEFORE THE FDA GETTING PRODUCT APPROVAL. AGAIN, THESE ARE ALL AVERAGE TIMES. FOR US, FOR EXAMPLE, THIS IS DATA THAT GOES BACK TO 1991. IT'S THE SAME FOR THE BIOPHARMA AND PHARMA INDUSTRY, SO IT'S ESSENTIALLY ABOUT 14 YEARS WORTH OF DATA THAT SIT BENEATH THESE. SO THESE ARE VERY LENGTHY PROCESSES ONCE YOU START CLINICAL TRIALS.

CHAIRMAN PENHOET: AND THE WORK THAT LED UP TO THOSE, STARTING THE CLINICAL TRIALS MIGHT BE AN EQUAL LENGTH OF TIME?

DR. JUELSGAARD: SO I'M GOING TO MAKE THAT POINT RIGHT NOW. I JUST TOOK TWO OF OUR MORE SUCCESSFUL PRODUCTS, BUT THEY'RE ALSO PRODUCTS WHICH COME FROM WHAT I CALL THE NEW BIOLOGICAL CONCEPT. LET ME JUST TALK ABOUT THOSE REAL QUICKLY. ONE IS HERCEPTIN, A DRUG TO TREAT METASTATIC BREAST CANCER. THE CONCEPT THERE WAS THAT YOU CAN TARGET AN ANTIBODY TO A CELL THAT OVEREXPRESSED, IN THIS CASE A CANCER CELL THAT OVEREXPRESSED, A CERTAIN PROTEIN, AND YOU COULD AFFECT THE CANCER, KILL THE CANCER, IF YOU WILL. IN ESSENCE, STEM CELLS ARE A BRAND NEW BIOLOGICAL CONCEPT. SO I

WANTED TO TAKE SOMETHING THAT YOU HAD TO REALLY SORT OF PROVE TO BE TRUE AS WELL AS DEVELOP THE PRODUCT. SO ESSENTIALLY THIS WAS A 14-YEAR EFFORT THAT WENT ON AT GENENTECH WITH RESPECT TO HERCEPTIN LEADING UP FROM THE BEGINNING TO THE END.

JUST TO SHOW YOU THAT THAT'S NOT A FLUKE, I TOOK THE LATEST PRODUCT THAT WE HAVE. SO THESE ARE OUR TWO LATEST PRODUCTS IN TERMS OF APPROVAL IN THE CANCER AREA. ANOTHER PRODUCT, ANOTHER NOVEL BIOLOGIC CONCEPT, AGAIN AN ANTIBODY, BUT THIS TIME NOT TARGETED AT A CANCER CELL, BUT TARGETED AT THE BLOOD SUPPLY THAT FEEDS CANCER TO TRY AND DOWN-REGULATE OR DEPRESS THE AMOUNT OF BLOOD SUPPLY AND THEREBY EITHER MODERATE OR KILL THE TUMOR GROWTH AS A RESULT OF INHIBITING ITS BLOOD SUPPLY. THIS WORK FROM THE TIME WE FIRST STARTED, AND ACTUALLY WORK STARTED SHORTLY AFTER I JOINED GENENTECH. I CAN REMEMBER WHEN NAPOLEON BRAR (PHONETIC) CAME ON BOARD AND SOME OF THE THINGS THAT HE WAS DOING AT THAT POINT IN TIME, BUT 16 YEARS ESSENTIALLY INVOLVED FROM THE POINT IN TIME WHEN YOU GET STARTED IN THE RESEARCH LAB UNTIL YOU COME OUT WITH A PRODUCT AT THE OTHER END.

SO IF THERE'S ONE THING TO TAKE AWAY FROM ALL OF THIS, YOU'RE TALKING ABOUT TEN YEARS OF FUNDING. SOMEBODY MADE THAT COMMENT EARLIER TODAY. THAT FUNDING IS ALL GOING TO BE LONG GONE AND OUT THE DOOR BEFORE THE

VERY FIRST THERAPY BECAUSE I THINK WE'RE STILL WAY BACK IN THE 1988 PHASE ON THIS SLIDE RIGHT NOW, IF EVEN THERE.

DR. PIZZO: ACTUALLY ON THIS EXAMPLE, IF YOU REALLY WANTED TO TRACK IT BACK TO THE FIRST FUNDAMENTAL CONCEPT, IT GOES BACK TO THE MID-1960S.

DR. JUELSGAARD: IT GOES BACK TO JUDITH FOLKMAN, AND IT'S SORT OF THE WHOLE THOUGHT PROCESS. I MOSTLY FOCUSED ON WHAT HAPPENED AT GENENTECH. IT JUST TAKES A TREMENDOUSLY LONG PERIOD OF TIME, AND THE REASON FOR THAT IS THAT THIS IS TOUGH BIOLOGY. BIOLOGY IS HARD AND IT'S GETTING HARDER. I THINK WE'VE GOT MOST OF THE EASY BIOLOGY OUT OF THE WAY. AND SO THE NEW PROBLEMS THAT WE'RE DEALING WITH ARE ONES THAT REALLY REQUIRE A FAIR AMOUNT OF EFFORT AND JUST A LOT OF HARD WORK. AND SO THE IDEA THAT THERE ARE GOING TO BE QUICK, EASY FIXES THAT COME OUT OF THIS, I HOPE PEOPLE UNDERSTAND ARE NOT LIKELY TO BE TRUE.

LET ME JUST SAY THIS. I DIDN'T MENTION THIS AT THE BEGINNING, BUT I'M HAPPY TO ENTERTAIN ANY QUESTIONS ALONG THE WAY AND OBVIOUSLY AT THE END.

SO THE THIRD THING IS JUST TO ADDRESS THE COST OF DRUG DEVELOPMENT. SO WE WANTED TO LOOK AT WHETHER WE AT GENENTECH WERE MORE EFFICIENT, LESS EFFICIENT, OR ABOUT AS EFFICIENT IN TERMS OF USING MONEY TO DEVELOP PRODUCTS AS THE REST OF THE INDUSTRY. AND IT TURNS OUT

THAT WE'RE A LITTLE MORE EFFICIENT. I DON'T WANT TO DWELL ON THAT BECAUSE WE TEND TO BE A LITTLE BIT MORE SUCCESSFUL, AND WE THINK WE'RE BEGINNING TO UNDERSTAND WHY, BUT THAT'S NOT REALLY THE POINT OF THIS.

THE POINT IS IS THAT THE COSTS THAT WE SEE HERE, I PARTICULARLY WANT TO POINT TO THE TOTAL ONE AT THE END OF THE DAY, IT COSTS US ON AN ALL-IN CAPITALIZED BASIS ABOUT $900 MILLION, CLOSE TO $1 BILLION, TO DEVELOP A SUCCESSFUL DRUG. THE INDUSTRY, THE DI MASI GROUP BROUGHT DATA FORWARD FOR THE REST OF THE INDUSTRY. REMEMBER, THEIR STUDY WAS DONE IN 1992 AND USED DATA THAT PRECEDED THAT. SO THEY BROUGHT THEIR DATA FORWARD FIVE YEARS BASED ON WHAT THEY UNDERSTOOD TO BE THE RATE OF APPRECIATION OF COST IN THE CLINICAL DEVELOPMENT AREA, WHICH IS ABOUT 17 PERCENT A YEAR. AND THEIR VIEW IS THAT THE LIKELY AVERAGE COST IN THE BIOPHARMA INDUSTRY RIGHT NOW TO DEVELOP A NEW PRODUCT IS IN THE NEIGHBORHOOD OF ABOUT $1.3 BILLION, COSTS ALL IN AND CAPITALIZED.

SO AGAIN, VERY EXPENSIVE PROPOSITIONS. AND SO THE AMOUNTS OF MONEY THAT IT TAKES REALLY TO BRING THESE PRODUCTS TO MARKET ARE PRETTY STAGGERING WHEN YOU CONSIDER, HENCE A COMMENT EARLIER ON, THAT FOR MANY YOUNG COMPANIES REALLY THE WAY THAT THEIR PRODUCTS ARE BROUGHT TO THE MARKET IS EITHER THROUGH LICENSING ARRANGEMENTS WITH BIGGER COMPANIES SO THAT THE PRODUCT AT SOME STAGE

GETS TURNED OVER TO A BIGGER COMPANY, LIKE A GENENTECH, TO MARKET, OR THEY GET ACQUIRED BY A BIG COMPANY. BUT AT THIS POINT IN TIME VERY, VERY FEW SMALL COMPANIES SORT OF MAKE IT TO THE POINT OF HAVING THEIR OWN PRODUCTS IN THE MARKETPLACE.

SO WHAT ARE THE TAKE-AWAY POINTS FROM WHAT I JUST PRESENTED? THERE ARE SORT OF THREE. AND I'VE ALREADY MADE THESE POINTS. ONE, THERE ARE GOING TO BE MANY FAILURES ALONG THE WAY. AGAIN, WHEN YOU'RE ESTABLISHING THE POLICIES AT CIRM THAT MAY DEAL WITH THE FOR-PROFIT WORLD OR THE PRIVATE ENTITY WORLD, THESE ARE ALL IMPORTANT FACTORS THAT I THINK YOU NEED TO THINK ABOUT BECAUSE THESE INFLUENCE HOW PEOPLE BEHAVE IN THE WORLD THAT I'M IN.

SO THERE ARE GOING TO BE A LOT OF FAILURES ALONG THE WAY. THERE ARE GOING TO BE SOME VERY LONG TIMES INVOLVED IN BRINGING NEW THERAPIES TO THE MARKETPLACE, AND IT'S GOING TO BE A VERY EXPENSIVE PROPOSITION. AND THE AMOUNT OF SUPPORT THAT THIS ORGANIZATION MIGHT PROVIDE ALONG THE WAY IS PROBABLY GOING TO BE, AS I PUT IT, THE PROVERBIAL DROP IN THE BUCKET COMPARED TO ALL THE COSTS THAT HAVE TO BE IDENTIFIED TO REALLY BE SUCCESSFUL. SO JUST PLANTING THOSE THOUGHTS FOR A MOMENT.

SO GENENTECH HAS OVER THE COURSE OF TIME HAD A NUMBER OF RELATIONSHIPS WITH GOVERNMENTAL INSTITUTIONS IN

A VARIETY OF AREAS. I'VE JUST SORT OF LISTED SOME OF THEM HERE AS FOR EXAMPLES. WE HAVE IN LICENSED INTELLECTUAL PROPERTY. I LISTED SORT OF THE ONE PREEMINENT ONES, THE COHEN BOYER PATENT, WHICH WAS THE GRANDFATHER PATENT IN THE WHOLE BIOTECH SECTOR, HAS NOW EXPIRED, BUT WE LICENSED THAT FROM STANFORD UNIVERSITY. ONE OF OUR CURRENT LICENSES, FOR EXAMPLE, AT THE UNIVERSITY OF IOWA A CMV PROMOTER THAT WE USE ON ONE OF OUR CELL LINES TO MAKE PRODUCTS. WE HAVE COLLABORATIVE RESEARCH AND DEVELOPMENT ARRANGEMENTS WHERE WE WORK WITH UNIVERSITIES JOINTLY ON RESEARCH. WE HAVE AN OPPORTUNITY. WE HAVE SUCH A MASTER AGREEMENT WITH UC THAT COVERS UC BERKELEY AND UCSF AND UC SANTA CRUZ, AND MORE RECENTLY DID ONE WITH UCLA. SO WE HAVE AGREEMENTS THAT COVER BEING ABLE TO WORK TOGETHER WITH INSTITUTIONS BECAUSE WE TRY TO DO SOME OF THAT.

AND THEN LASTLY, WE HAVE CONTRACT ARRANGEMENTS WITH GOVERNMENTAL AGENCIES, PARTICULARLY IN OUR CASE WITH THE NIH. WE CONTRACT WITH THEM TO DO CLINICAL TRIAL WORK. THEY HAVE A HUGE CLINICAL TRIAL INFRASTRUCTURE SET UP IN THE ONCOLOGY AREA, SEVERAL GROUPS INVOLVED, AND THEY'RE VERY KEEN ON DOING CLINICAL TRIAL WORK AROUND NOVEL CANCER THERAPIES.

FUNDED RESEARCH, WE DON'T GET INVOLVED IN FUNDING FROM GOVERNMENT INSTITUTIONS. AND PART OF THAT

REALLY RELATES TO SOME OF THE ISSUES, WHICH I'M GOING TO COME TO NEXT AND WHICH HAVE ALREADY BEEN ALLUDED TO, BECAUSE OF SOME OF THE CONSTRAINTS THAT COME WITH GOVERNMENT FUNDING AT THE END OF THE DAY THAT JUST DON'T WORK AT LEAST, FOR US AS AN ORGANIZATION, AND I THINK ARE PROBABLY SEEN AS PROBLEMATIC BY A NUMBER OF ENTITIES ON THE PRIVATE SIDE.

SO WHAT ARE SOME OF THOSE RED-FLAG PROVISIONS? AGAIN, I'M EXPRESSING MY POINT OF VIEW. SO I'M NOT HERE ON BEHALF OF THE GOVERNMENT OR TAXPAYERS OR ANYBODY ELSE, BUT IN THE BUSINESS WORLD, WHAT ARE THESE THINGS THAT RAISE FOR US CONCERNS? AND I LISTED SORT OF THREE. THERE ARE OTHERS THAN THESE, BUT THESE ARE THREE OF THE MAJOR ONES.

ONE ARE FAIR OR REASONABLE PRICE PROVISIONS. HOPEFULLY THESE ARE SELF-EVIDENT. I'D BE HAPPY TO TALK A LITTLE BIT ABOUT THEM MORE LATER.

MARCH-IN RIGHTS PROVISIONS, PARTICULARLY WHEN THEY INVOLVE COMING IN AND TAKING OVER A PROJECT OR INSERTING SOMEBODY ELSE IN DEALING WITH THE PROJECT. AND THEN REQUIREMENTS THAT SOMETHING THAT MAY RESULT FROM WORK THAT'S DONE BE LICENSED TO OTHERS ON A NONEXCLUSIVE BASIS SO THAT THERE'S SOME OPPORTUNITY FOR NONEXCLUSIVITY FOR THE COMPANY THAT'S INVOLVED NOT TO HAVE AN EXCLUSIVE POSITION WITH RESPECT TO THOSE ARRANGEMENTS.

WHY ARE THESE RED FLAGS FOR US? WELL, ONE OF THE THINGS THAT WE TRY TO DO IN THE BUSINESS WORLD IS WORK DOWN THE UNCERTAINTY LADDER. WE'RE TRYING TO WORK FROM A HUGE AMOUNT OF UNCERTAINTY, WE HAVE SCIENTIFIC UNCERTAINTY, WE HAVE FINANCIAL UNCERTAINTY, LEGAL UNCERTAINTIES. AND AT THE END OF THE DAY, WE'RE TRYING TO ELIMINATE UNCERTAINTIES RATHER THAN ENLARGE THEM. AND ALL THIS DOES IS TEND TO CREATE THE POSSIBILITY OF HAVING MORE UNCERTAINTY IN THE FUTURE. WHEN SOMEBODY EXERCISES A MARCH-IN RIGHT, WHAT ABOUT THE NOTION OF SOMEBODY WANTING TO BECOME INVOLVED IN YOUR PRICING DECISIONS? SO FROM A PURE BUSINESS MODEL, THIS SORT OF IS WORKING IN THE OPPOSITE DIRECTION OF TRYING TO ELIMINATE THOSE UNCERTAINTIES AS YOU PROGRESSIVELY WORK TOWARDS A PRODUCT OUTCOME.

THE SECOND IS THAT SOME OF THESE PROVISIONS GO WELL BEYOND, AT LEAST IN MY VIEW, THE SCOPE OF THE VALUE THAT YOU'RE GETTING IN RETURN. REMEMBER, I INDICATED THAT IT'S GOING TO COST US AROUND $900 MILLION TO DEVELOP A PRODUCT, 1.3 BILLION IF YOU BELIEVE THE BROUGHT FORWARD DI MASI DATA. AND YOU HEARD ABOUT THE SIZE OF SOME OF THE GRANTS THAT HAVE BEEN MADE EITHER BY THE GOVERNMENT OR BY THE JUVENILE DIABETES FOUNDATION. THE AMOUNTS OF MONEY THAT WE'RE TALKING ABOUT ARE PALE IN COMPARISON TO THE GRAND SCHEME OF ALL THE MONEY THAT GOES IN. SO THE

NOTION THAT FOR WHAT I CONSIDER OR WHAT MAY BE SEEN AS A DROP IN THE BUCKET TO HAVE ESSENTIALLY THE RIGHT TO COME IN AND TAKE A PROGRAM AWAY OR PROVIDE IT TO SOMEBODY ELSE TO HELP YOU SET YOUR PRICE, ETC., IS FROM MY POINT OF VIEW A PRETTY DISPROPORTIONATE SET OF CIRCUMSTANCES.

AND THEN THE THIRD IS JUST A LOT OF THE ISSUES THAT GO ON WITHIN A COMPANY ARE REALLY GERMANE TO THAT COMPANY. THERE'S A LOT OF WEIGHING AND BALANCING. COMPANIES ARE NOT FOCUSED ON ONE THING, HOPEFULLY NOT. FOR THE MOST PART, THEY'RE BALANCING AND JUGGLING A NUMBER OF PROJECTS AND PROPOSITIONS AT THE SAME TIME TRYING TO MAKE THE ENTERPRISE WORK. AND SO WHAT HAPPENS INSIDE A COMPANY IS USUALLY WELL-KNOWN TO THE PEOPLE WITHIN THE COMPANY AND GERMANE TO THAT COMPANY, AND TO BRING OUTSIDERS INTO THAT ENTERPRISE TO HELP MAKE JUDGMENTS ABOUT WHAT IT SHOULD DO WHO ARE ILL-EQUIPPED TO REALLY UNDERSTAND THE BROAD SCOPE OF WHAT'S GOING ON AROUND THE ISSUES OF PACE OF DEVELOPMENT OF A PRODUCT OR PRICING DECISIONS OR WHATEVER JUST IS A VERY, VERY ODD FIT.

SO FROM OUR POINT OF VIEW, WE WOULD -- SHY AWAY IS NOT A STRONG ENOUGH TERM. WE SIMPLY WOULDN'T ENGAGE IN ARRANGEMENTS WITH ORGANIZATIONS IF ANY OF THESE WERE CONSIDERATIONS SIMPLY BECAUSE OF THE DOWNSTREAM LIMITATIONS THAT POTENTIALLY COME WITH THEM. IT'S JUST

NOT A RISK WORTH TAKING BECAUSE WE BELIEVE WE CAN ADDRESS OUR NEEDS IN OTHER WAYS.

I WANT TO THEN SWITCH GEARS JUST TO TALK A LITTLE BIT ABOUT THE TYPICAL PRIVATE SECTOR FUNDING ARRANGEMENTS, AND WHAT I MEAN BY THAT IS USUALLY WHAT GOES ON IN THE PRIVATE SECTOR INDUSTRY BECAUSE I THINK THAT THERE ARE MODELS OUT THERE OBVIOUSLY THAT ONE CAN USE IN TERMS OF THINKING ABOUT IF ONE IS GOING TO PROVIDE FUNDING, WHAT THE RETURNS MIGHT BE. THERE'S NOTHING REALLY TERRIBLY ILLUMINATING ABOUT THIS OTHER THAN THESE ARE THE ONES AT LEAST THAT WE SEE MOST COMMONLY AND WE SOMETIMES GET INVOLVED IN, PARTICULARLY WHERE WE LEND MONEY OR WHERE WE MAKE INVESTMENTS IN OTHER COMPANIES. WE TEND TO BE ON THE FUNDING SIDE IN MOST OF OUR ARRANGEMENTS WITH OTHER COMPANIES IN THIS DAY AND AGE.

SO A VERY SIMPLE APPROACH. ONE IS A LOAN ULTIMATELY TO BE PAID WITH MARKET INTEREST RATES. WE HAVE A COUPLE OF ARRANGEMENTS WITH COMPANIES THAT HAVE DEVELOPED PRODUCTS OF OURS THAT WE HAVE A ROLE IN WHERE WE'VE SIMPLY LENT THEM THE MONEY WITH THE AGREEMENT THAT THEY'LL PAY US BACK OVER TIME LATER ON, OBVIOUSLY ALWAYS SUBJECT TO CREDIT RISK.

ANOTHER APPROACH VERY SIMILAR TO THE FIRST ONE, BUT IS A LOAN ESSENTIALLY WITH THE REPAYMENT TO BE MADE DOWNSTREAM IF THE PRODUCT IS SUCCESSFUL BASED ON A

ROYALTY RATE, A MUCH RISKIER PROPOSITION BECAUSE THERE'S NO GUARANTEE OF REPAYMENT, ONLY IF THE PRODUCT'S SUCCESSFUL, BUT ANOTHER POTENTIAL WAY OF ADDRESSING PROVIDING FUNDING AND GETTING A RETURN.

OR THE THIRD, WHICH IS AGAIN SOMEWHAT COMMON, PROVIDING LOANS WHICH CAN BE REPAID IN EQUITY EITHER AT THE BORROWER'S DISCRETION OR AT THE LENDER'S DISCRETION, SO DEBT CONVERTIBLE INTO EQUITY BASED ON SOME PREESTABLISHED CRITERIA.

THE OTHER, WHICH IS A COMMON VENTURE CAPITAL TECHNIQUE, BUT ALSO EMULATED IN THE PRIVATE WORLD, GENENTECH'S WORLD, FOR EXAMPLE, IS IF WE MAKE INVESTMENTS IN OTHER COMPANIES TO DEVELOP PRODUCTS, ONE OF THE THINGS THAT WE GET ALONG WITH PRODUCT RIGHTS ARE EQUITY OR STOCK OR OTHER FORMS OF EQUITY COMPENSATION AS PART OF PUTTING THAT MONEY IN. SO IN TERMS OF THINKING ABOUT IF ONE WERE -- IF CIRM WERE TO PUT MONEY INTO PRIVATE ENTITIES, WAYS OF THINKING ABOUT WHAT SORT OF PAYBACK THERE MIGHT BE, THESE ARE ONES THAT I WOULD OBVIOUSLY LOOK AT BECAUSE THESE ARE VERY WELL-KNOWN, VERY WELL-UNDERSTOOD IN THE PRIVATE SECTOR, A LOT OF PRIOR KNOWLEDGE TO BE PIGGYBACKED ON.

LET ME JUST -- A DIVERSION FOR A MOMENT BECAUSE THIS QUESTION ACTUALLY GOT ASKED, AND MARY ASKED THAT I ADDRESS IT AS WELL, AND THIS HAS TO DO WITH ACCESS TO

THERAPY, WHICH IS AN INCREASING ISSUE IN THIS COUNTRY AND OBVIOUSLY OF GREAT CONCERN AND GROWING CONCERN AS THE PRICES OF NEW THERAPIES INCREASE DRIVEN IN LARGE PART BY THE COST OF NEW INNOVATION.

SO IN THE WORLD THAT I DEAL IN, MOST OF THE PHARMACEUTICAL AND BIOTECHNOLOGY COMPANIES HAVE PROGRAMS EXISTING IN PLACE THAT THEY'VE HAVE HAD FOR SOME TIME NOW, PROGRAMS TO TRY AND ADDRESS WHAT WE CALL ACCESS TO CARE; THAT IS, ACCESS FOR THOSE PEOPLE WHO FIND IT DIFFICULT TO FIND ACCESS TO THERAPY. GENERALLY SPEAKING, ACCESS ISSUES ARISE IN ONE OF THREE WAYS. ONE, YOU DON'T HAVE ANY HEALTH INSURANCE AT ALL. TWO, YOU DO HAVE HEALTH INSURANCE, BUT IT JUST DOESN'T COVER THIS PARTICULAR THERAPY THAT THE DOCTOR HAS PRESCRIBED FOR YOU. OR THREE, YOU DO HAVE THE HEALTH INSURANCE, IT DOES COVER THE THERAPY, BUT THE COPAYMENTS ARE SO LARGE THAT YOU SIMPLY CAN'T AFFORD IT AT THE PERSONAL LEVEL.

SO THOSE ARE, AT LEAST AS WE SEE IT, THE THREE MAJOR ACCESS ISSUES TO BE DEALT WITH. WE'VE TAKEN A PARTICULAR APPROACH. AGAIN, I DON'T WANT TO DWELL ON WHAT GENENTECH DOES EXCEPT TO POINT OUT THAT THERE ARE WAYS THAT COMPANIES TRY TO ADDRESS THESE ISSUES AROUND THEIR OWN PRODUCTS. SO WE HAVE SOMETHING CALLED THE ACCESS TO CARE FOUNDATION, WHICH ESSENTIALLY TRIES TO ADDRESS THE FIRST OF THOSE TWO ISSUES, PEOPLE THAT EITHER

DON'T HAVE INSURANCE OR HAVE INSURANCE THAT DOESN'T COVER OUR PRODUCT. AND WHAT WE DO IN THOSE CASES IS THAT WE PROVIDE FREE PRODUCT TO THOSE PATIENTS, THE PRODUCT THAT THE INSURANCE WON'T TAKE CARE OF.

LAST YEAR, AND WE FIND THIS A GROWING AREA, LAST YEAR WE PROVIDED IN THE NEIGHBORHOOD OF $200 MILLION WORTH OF FREE PRODUCT BASICALLY TO PEOPLE WHO FIT THAT MODEL. OVER THE COURSE OF TIME, SINCE WE INSTITUTED THIS PROGRAM, WE'VE PROVIDED ABOUT $700 MILLION WORTH OF FREE PRODUCT.

THE SECOND, AND THIS IS ANOTHER GROWING AREA, IS AS YOU SEE THE TREND TO ENLARGE THE COPAYMENTS THAT PEOPLE HAVE TO MAKE TO GET HEALTHCARE, AND THAT'S A GROWING PHENOMENON, WE ARE LIMITED IN OUR ABILITY TO PROVIDE COPAYMENT SUPPORT BECAUSE OF GOVERNMENTAL RULES AND REGULATIONS AND THINGS, PROHIBITIONS. BUT INSTEAD, WHAT'S HAPPENED IS THAT THIRD-PARTY INSTITUTIONS, CHARITABLE ORGANIZATIONS, HAVE STARTED TO ARISE OUT OF THE ARENA WHO WILL ON A NEEDS BASIS USUALLY THESE ORGANIZATIONS PROVIDE SUPPORT TO PEOPLE UP TO -- WHO ARE AT THE THREE TO FOUR TIMES POVERTY LEVEL INCOME, UP TO THAT LEVEL, PROVIDING COPAYMENT ASSISTANCE SUPPORT FOR PARTICULAR SORTS OF MEDICAL PROBLEMS THAT THEY MIGHT HAVE, THEY'RE USUALLY PROVIDED, NOT FOR PARTICULAR THERAPIES, BUT FOR THE TREATMENT OF MEDICAL CONDITIONS.

SO WITH THE ADVENT OF THOSE, WE HAVE BEGUN FUNDING THESE SORTS OF ORGANIZATIONS TO HELP WITH THAT PARTICULAR KIND OF ACCESS ISSUE; THAT IS, THE RISING COST OF COPAYMENTS. SO MY POINT IS THAT THE PRIVATE SECTOR IS HELPING OR TRYING TO HELP SERVE THESE ISSUES IN TERMS OF ACCESS. IT HAS VARIOUS PROGRAMS. THESE ARE OURS. AND I THINK IT'S VERY REASONABLE TO HAVE EXPECTATIONS OF THE PRIVATE SECTOR TO ADOPT OR PUT IN PLACE PROGRAMS OF THIS SORT AS WE MOVE FORWARD. AND I THINK THESE CAN BE DONE IN LIEU OF SOME OF THE OTHER POTENTIAL WAYS OF ADDRESSING THESE ISSUES.

SO I JUST COME TO MY -- THESE ARE MY RECOMMENDATIONS FOR WHAT THEIR WORTH, IF YOU WILL, AT THE END OF THE DAY. IF THE DECISION IS MADE BY CIRM TO PROVIDE FUNDING OR SUPPORT FOR THE PRIVATE SECTOR ORGANIZATIONS, I WOULD ENCOURAGE YOU REALLY TO THINK ABOUT SORT OF THE EXISTING MECHANISMS AND UTILIZING THOSE SINCE THEY'RE WELL-WORN, WELL-UNDERSTOOD, AND WILL BE MUCH EASIER TO DEAL WITH AND ADMINISTER THAN OTHERS.

I WOULD BE VERY LOATHE TO IMPOSE NONFINANCIAL CONSTRAINTS IN ANY OF THESE BECAUSE IF YOUR GOAL, IF THE GOAL IS, THIS IS AGAIN MY VIEW, IF THE GOAL IS TO REALLY ADVANCE SCIENTIFIC INNOVATION IN THIS AREA, COMPANIES, PRIVATE COMPANIES, HAVE A LARGE ROLE AND AN IMPORTANT ROLE TO PLAY IN IT, BUT THESE WILL CREATE IMPEDIMENTS FOR

A NUMBER OF COMPANIES TO GET INVOLVED. THEY WILL SIMPLY LOOK ELSEWHERE TO DO WHAT THEY NEED TO DO TO AVOID THESE SORTS OF POTENTIAL DOWNSTREAM ISSUES.

IN MY VIEW, WHAT WILL HAPPEN WITH THESE IS YOU WILL INSTEAD PUSH THE FUNDING TO ACTUALLY THE RISKIEST OF THE ENTERPRISES. SO THE ONES THAT ARE MORE LIKELY TO WORK, THE ONES THAT HAVE THE GREATEST CHANCE OF SUCCESS, PEOPLE WILL NOT WANT TO ENCOUNTER THESE POTENTIAL DOWNSTREAM RISKS, SO THEY WILL LOOK FOR OTHER WAYS TO SOLVE WHAT THEY NEED. INSTEAD THE PEOPLE WHO WILL COME ASKING OR LOOKING FOR HELP ARE GOING TO BE THE ONES THAT ARE THE FARTHEST OUT ON THE RISK SPECTRUM. AND I THINK THERE'S A TENDENCY THAT YOU'D WIND UP SUPPORTING THOSE SORTS OF ENTERPRISES.

AND THEN LASTLY, I DO THINK ACCESS TO HEALTHCARE AND ACCESS TO TREATMENT IS AN IMPORTANT ISSUE. IT'S A SOCIETAL ISSUE. IT'S SOMETHING THAT WE NEED TO ADDRESS. THERE ARE WAYS OF ADDRESSING IT, AND I THINK IT'S IMPORTANT TO PUT SOMETHING IN PLACE. AND I'VE GIVEN YOU AN EXAMPLE OF AT LEAST ONE WAY OF DOING IT. BUT I WOULD ENCOURAGE SOLVING THAT PROBLEM IN THE WAY OR FASHION THAT I'VE IDENTIFIED AS OPPOSED TO ADDRESSING ISSUES LIKE HOW COMPANIES PRICE PRODUCTS, ETC. SO LET ME END THERE. I THINK THAT'S THE END OF THE PRESENTATION. IF YOU HAVE ANY QUESTIONS.

CHAIRMAN PENHOET: THANK YOU VERY MUCH.

MR. SHEEHY: WELL, I HAD COUPLE OF A QUESTIONS, NOT NECESSARILY RELATED TO EACH OTHER. BUT I WAS INTERESTED IN ONE OF YOUR EARLIER SLIDES ON PROTEIN AND PEPTIDE PATENTS. HHS ACTUALLY HOLDS SOME PATENTS?

DR. JUELSGAARD: ACCORDING TO THE -- THIS DATA ALL COMES FROM THE U.S. PATENT AND TRADEMARK OFFICE, AS I SAID. SO THIS IS THE DATA THAT'S TRANSLATED DIRECTLY FROM THEM. THIS IS SOMETHING THAT WAS PUT TOGETHER BY OUR PATENT GROUP AT GENENTECH, TO BE HONEST WITH YOU, SO I HAVEN'T DUG UNDERNEATH THAT.

DR. PIZZO: BUT IF YOU'RE AN NIH INVESTIGATOR, YOU COULD SPEAK TO THIS; BUT IF YOU'RE AN INTRAMURAL NIH INVESTIGATOR AND YOU HAVE A DISCOVERY, YOU CAN ACTUALLY HOLD A PATENT FOR THAT. THAT'S PROBABLY --

MR. SHEEHY: I WAS JUST CURIOUS BECAUSE WE'VE BEEN TOLD ALL ALONG THAT THE FEDERAL GOVERNMENT DOESN'T TRY TO HOLD PATENTS.

CHAIRMAN PENHOET: WHEN THEY'RE INVENTED BY FEDERAL GOVERNMENT EMPLOYEES, NOT WHEN THEY'RE BY GRANTEES.

MR. SHEEHY: JUST CURIOUS. JUST JUMPED OUT AT ME.

THE OTHER THING I HAD A QUESTION, THE RED FLAGS AND MARCH-IN RIGHTS. THESE ARE ALL PART OF BAYH-DOLE,

AND TYPICALLY IT SEEMS TO ME THAT A LOT OF YOUR BASIC RESEARCH DISCOVERIES ARE FUNDED THROUGH THE FEDERAL GOVERNMENT AND BAYH-DOLE. SO THAT DOESN'T DISAPPEAR WHEN THEY LICENSE WITH YOU. IF WE HAVE SIMILAR PROVISIONS, WHY ARE THOSE SUCH RED FLAGS WHEN THEY'RE ALREADY PART OF THE ENVIRONMENT IN WHICH YOU'RE WORKING?

DR. JUELSGAARD: WELL, IT DEPENDS. SO THERE ARE TWO POINTS. IT DEPENDS, FIRST OF ALL, ON WHAT THE MARCH-IN RIGHTS RELATE TO. THE SIMPLE MARCH-IN RIGHTS ARE, YOU KNOW, WE WANT TO HAVE A PATENT APPLICATION FILED. I DON'T HAVE ANY ISSUE WITH THAT BECAUSE, GENERALLY SPEAKING, AS AN ORGANIZATION, IF WE DECIDE NOT TO FILE A PATENT APPLICATION ABOUT SOMETHING, WE GENERALLY BELIEVE THERE'S NO INVENTION THERE, IT'S NOT WORTH IT. IF SOMEBODY ELSE WANTS TO FOLLOW IN OUR WAKE, THAT'S FINE.

SO IT REALLY DEPENDS ON THE LEVEL. WHEN I SAY MARCH-IN RIGHTS, THAT'S A BIG CATEGORY. MY BIGGER CONCERN, THIS IS ACTUALLY ONE THAT WE FACED WITH THE NIH WAY BACK WHEN, WE HAD AN ARRANGEMENT WITH MARCH-IN RIGHTS RELATED TO A COMPOUND THAT WE WERE STUDYING. AND THE NIH WASN'T HAPPY WITH THE PROGRESS THAT WE WERE MAKING. IT WAS A VERY, VERY DIFFICULT COMPOUND TO STUDY, AND THEY HAD EXPECTATIONS OF HOW LONG IT WOULD TAKE TO DO THIS, AND WE WEREN'T MEETING THEIR EXPECTATIONS.

AND SO WHAT WE WOUND UP DOING IS CHANGING THE EXCLUSIVE ARRANGEMENT TO A NONEXCLUSIVE ARRANGEMENT WITH THEM, AND THE ULTIMATE END RESULT OF THAT WAS WE JUST DECIDED TO HECK WITH THIS. WE'LL SPEND OUR MONEY ELSEWHERE. WE'LL WORK ON THINGS WHERE WE UNDERSTAND OUR ECONOMIC POSITION BETTER THAN THIS ONE. SO, YES, THEY EXIST. THEY'RE THERE. DEPENDS ON THE SCOPE OF THEM. I TAKE IT THAT THE NIH HAS HARDLY EVER INSTITUTED THEM, AND THERE'S SOME CONFIDENCE IN TERMS OF HOW THE NIH REACTS, BUT THAT DOESN'T MEAN THAT EVERY ORGANIZATION WILL FACE IT IN THE SAME WAY. EVERY TIME A NEW ORGANIZATION APPEARS WITH MARCH-IN RIGHTS, I THINK YOU HAVE TO REALLY TAKE A STEP BACK AND LOOK VERY KEENLY AT WHAT MIGHT HAPPEN DOWN THE ROAD, BUT I WOULDN'T PRESUME THAT EVERYBODY WILL BEHAVE THE SAME.

SO I HAVE SOME FAIR COMFORT WITH THE NIH ALTHOUGH WE DID HAVE AN EXPERIENCE THAT SORT OF LED US NOT TO DEAL WITH THESE ISSUES ANYMORE.

MR. SHEEHY: I JUST WAS THINKING IN TERMS OF THE IP RULES THAT WE PUT IN FOR NONPROFITS. IF THERE'S ANY INTERFERENCE, SO TO SPEAK, WITH THE ABILITY OF A COMPANY LIKE GENENTECH WITH STANFORD ASSUMING YOU GOT A GRANT FROM US.

DR. JUELSGAARD: THOSE PRESENT PROBLEMS. EVERY ONCE IN A WHILE WE'LL RUN INTO ARRANGEMENTS THAT WE DO

WITH OTHER COMPANIES WHERE WE'RE TRYING TO BRING SOMETHING IN, AND THEY WILL HAVE AN ARRANGEMENT WITH AN INSTITUTION WHERE THERE ARE MARCH-IN RIGHTS, AND IT BECOMES A THORNY ISSUE. UNTIL WE CAN GET THAT RESOLVED AND CREATE MORE CERTAINTY AROUND IT, WE MAY NOT WIND UP DOING ANYTHING.

MR. SHEEHY: IT SEEMS LIKE SO MUCH BASIC RESEARCH IS FUNDED BY THE FEDERAL GOVERNMENT.

DR. JUELSGAARD: TRUE, BUT IT PROBABLY ISN'T NECESSARY TO LICENSE IT. THERE AREN'T THAT MANY FUNDAMENTAL PATENTS AT THE END OF THE DAY THAT COME OUT OF BASIC RESEARCH, YOU KNOW, THAT REALLY GIVE RISE. THESE ARE MORE TOOLS AND TECHNIQUES. THE REAL PATENTS THAT ARE IMPORTANT ARE GOING TO BE COMPOSITION OF MATTER PATENTS, USE PATENTS, THINGS OF THAT SORT. THAT'S WHAT WE RELY ON.

PATENTS I GAVE YOU THAT ARE EXAMPLES HERE, THEY ARE REALLY WAYS OF DOING THINGS, TECHNOLOGY, COHEN BOYER, CMV PROMOTER. FUNDAMENTALLY WE'RE NOT USING A LARGE RESERVOIR. WHEN I ASKED, GIVE ME EXAMPLE OF PATENTS THAT COME OUT OF ACADEMIA THAT WE'RE REALLY LICENSING AND USING THESE DAYS, THE LIST WAS PRETTY SHORT.

MR. SHEEHY: ONE MORE. SORRY. NOW, THE NIH PRESENTATION WE HAD EARLIER REALLY SHOWED A REAL STRONG BIAS TOWARDS SMALL BUSINESS. I JUST WONDER,

NOTWITHSTANDING THE ECONOMIC DEVELOPMENT ISSUE, GIVEN THE STATE OF THE SCIENCE, IS THAT REALLY A BIGGER BANG FOR OUR BUCK THAN ACTUALLY TRYING TO CAPTURE SOMEBODY LIKE GENENTECH IN THE RULES THAT WE WRITE?

DR. JUELSGAARD: I THINK THAT'S A GOOD QUESTION. I THINK THAT'S REALLY ONE THAT YOU HAVE TO ASK YOURSELF IS WHERE ARE YOU GOING TO PUT YOUR BETS AT THE END OF THE DAY, IN THE SMALL COMPANY WORLD OR MORE GLOBALLY THAN THAT? A POINT I TRIED TO MAKE IS IT'S HIGHLY UNLIKELY THAT EVEN IF YOU LIMITED YOURSELF TO INVESTING IN SMALL COMPANIES, AT THE END OF THE DAY, YOU'LL WIND UP AT THE END LINE JUST WITH A LIST OF SMALL COMPANIES BECAUSE THEY ARE EITHER GOING TO HAVE TO ENTER INTO LICENSING AGREEMENTS BECAUSE OF THE AMOUNT OF FUNDING THAT'S REQUIRED. LARGE COMPANIES WILL BE ACQUIRED BY LARGE COMPANIES. SO THERE WILL BE INEVITABLY MAJOR PLAYERS INVOLVED IN THESE ALONG THE WAY.

MR. SHEEHY: YEAH, BUT I'M JUST THINKING IN TERMS OF INVESTMENT. WOULDN'T IT BE A BETTER OBJECT FOR US TO LET YOU GUYS COME IN AND BUY THE COMPANY THAT WE GOT STARTED THAN TO TRY TO DEAL WITH YOU AT THE FRONT END?

DR. JUELSGAARD: WELL, THE QUESTION WHAT -- YES, ASSUMING THAT --

MR. SHEEHY: YOUR MAIN INVESTMENT IS GOING TO BE

THE LARGE --

DR. JUELSGAARD: ASSUMING THAT THE TERMS ARE RIGHT AND THAT WE'RE WILLING TO BUY UNDER THOSE CIRCUMSTANCES, RIGHT.

CHAIRMAN PENHOET: IF I COULD, MAYBE A POINT THAT YOU MADE RIGHT AT THE BEGINNING OF THE TALK WILL BE IMPORTANT FOR US, PRIORITIES. IF YOU WANTED TO -- IF YOU WANT THE MONEY TO BE USED TO SPAWN LOTS OF SMALL COMPANIES, YOU MIGHT DIRECT IT THERE. IF YOU WANT THE MONEY TO BE MOST EFFECTIVELY UTILIZED TO DEVELOP THERAPIES, YOU'D PROBABLY BET ON AN ORGANIZATION WHICH IS VERY GOOD AT DOING THAT. THAT MAY NOT BE THE SAME UNIVERSE OF COMPANIES.

MR. SHEEHY: EXCEPT WE'RE NOT AT A POINT IN SCIENCE WHERE THERE ARE THERAPIES NEAR DEVELOPMENT.

CHAIRMAN PENHOET: I THINK IT WAS AN IMPORTANT POINT.

DR. PIZZO: I'M GOING TO FOLLOW SOME OF JEFF'S QUESTIONS. I THINK THEY WERE VERY GOOD ONES. JUST TO BEGIN, I AGREE WITH YOU THAT THE NUMBER OF PATENTS THAT YIELD LARGE DOLLARS TEND TO BE VERY SMALL FROM ACADEMIA, AND THEY'RE USUALLY TECHNOLOGY PLATFORM KINDS OF THINGS. THAT IS TRUE WITH COHEN BOYER AND IT'S TRUE WITH ONE THAT WE HAVE NOW THAT'S A SIGNIFICANT ONE, BUT IT'S AMONG MANY, MANY, MANY THAT HAVE BEEN SUBMITTED.

GIVEN THE CAVEATS THAT YOU PUT FORWARD IN WHAT WAS A REALLY HELPFUL PRESENTATION, STEVE, THANK YOU, THAT YOU ARE NOT SPEAKING FOR GENENTECH, THAT GENENTECH IS NOT INVOLVED IN STEM CELL RESEARCH, AND THAT YOU'RE OFFERING PERSPECTIVES ON HOW THIS MIGHT ALL WORK. I TOOK HEART IN ONE OF THE POINTS THAT JEFF RELATED TO WHICH WAS YOUR SECOND BULLET IN YOUR RED FLAGS ABOUT EXCLUSIVITY, MARCH-IN RIGHTS, AND THE LIKE.

TO WHAT DEGREE DO YOU THINK OTHER BIOTECH ORGANIZATIONS OTHER THAN GENENTECH MIGHT BE RESISTANT OR CONCERNED ABOUT SOME OF THOSE SAME ISSUES BECAUSE THEY'RE GOING TO HAVE AN IMPACT ON US AS WE MOVE FORWARD?

DR. JUELSGAARD: I THINK IT WOULD BE DISINGENUOUS FOR ME TO SUGGEST THAT EVERYBODY BELIEVES AS I DO. I THINK THERE'S CERTAINLY A RANGE OF OPINION OUT THERE. AND AT THE END OF THE DAY, IT'S A TRADE-OFF. SO WHAT COMES WITH THOSE MARCH-IN RIGHTS IS FUNDING AND WHATEVER OTHER BENEFITS. AND IT REALLY DEPENDS UPON YOUR RISK TOLERANCE FOR THOSE SORTS OF RIGHTS. ON THE ONE HAND, TO THE EXTENT THAT YOU'LL SEE THEM DOWN THE ROAD AND THEY'LL IMPACT HOW YOU DO BUSINESS VERSUS YOUR NEED FOR THE FUNDING OR WHATEVER ELSE IS TO BE PROVIDED. SO I'M SURE THAT -- I CAN'T HAVE GIVE YOU A GOOD ANSWER, PHIL, BECAUSE I'VE NOT DONE A SURVEY AND I DON'T KNOW. PROBABLY BE A GREAT QUESTION TO ASK, TO REALLY GO OUT AND

MAYBE ASK A NUMBER OF COMPANIES AS A PROJECT.

DR. PIZZO: I THINK THAT WOULD BE HELPFUL FOR US TO DO TO KIND OF GAUGE THE TEMPERATURE OF THE COMMUNITY BECAUSE WE ARE FORTUNATE TO BE SURROUNDED BY OUTSTANDING BIOTECH COMPANIES, AND WE OUGHT TO AT LEAST KNOW. AND THEN WE HAVE TO MONITOR WHAT THEY FEED BACK TO US AS WELL, SO WE OUGHT TO KNOW WHERE THEY ARE, AND THEN WE'LL HAVE TO MAKE SOME ASSESSMENTS AS TO --

DR. JUELSGAARD: IT'S AT LEAST WORTH ASKING THE QUESTION AND SEEING HOW PEOPLE FEEL ABOUT IT.

CHAIRMAN PENHOET: OTHER QUESTIONS FROM THE TASK FORCE HERE IN SAN FRANCISCO? IF NOT, ANY QUESTIONS FROM FRANCISCO AT THE SUTTER MEDICAL PLAZA?

DR. PRIETO: I THINK JEFF COVERED MOST OF THE QUESTIONS THAT I HAD.

CHAIRMAN PENHOET: JANET WRIGHT IN CHICO.

(INTERRUPTION IN PROCEEDINGS.)

DR. FONTANA: I HAVE SOME QUESTIONS. IT'S JEANNIE FONTANA FROM L.A. I'D LIKE TO REFER TO YOUR SLIDE NO. 9 WHERE YOU SHOW THE PROBABILITY OF CLINICAL APPROVAL SUCCESS RATE FOR GENENTECH. I WAS CURIOUS TO WHAT DO YOU ATTRIBUTE GENENTECH'S APPROVAL SUCCESS RATE AND ANY OF THOSE ATTRIBUTES YOU THINK WE SHOULD INCORPORATE INTO CIRM'S SCIENTIFIC STRATEGIC PLAN?

DR. JUELSGAARD: WELL, AS I SAID, I REALLY

DIDN'T INTEND IN THIS PRESENTATION TO GET INTO WHAT HAPPENS AT GENENTECH AND USED THESE MOSTLY BECAUSE THEY WERE READILY AVAILABLE TO ME TO MAKE A BROADER POINT.

TO ANSWER YOUR QUESTION REALLY QUICKLY, WHY DO I THINK THAT WE'RE PERHAPS A LITTLE BIT MORE SUCCESSFUL, AT LEAST AT THIS TIME, AT GENENTECH, I THINK IT REALLY RELATES TO TWO THINGS THAT WE TRY TO IDENTIFY, ONE OF WHICH IS THAT WE'RE VERY MUCH A SCIENCE-DRIVEN ORGANIZATION. SO WE REALLY TRY TO SPEND A LOT OF TIME UNDERSTANDING THE SCIENCE THAT UNDERLIES -- THE BIOLOGICAL MECHANISMS OF ACTION AND THEN WHAT IMPACT THEM. AND WE HAVE, AS I ALLUDED TO EARLIER, WE HAVE A PHENOMENAL RESEARCH ORGANIZATION AT GENENTECH, A LARGE NUMBER WHO CAME OUT OF ACADEMIA, INCLUDING SOME OF THE GREAT CENTERS HERE IN THE BAY AREA.

SO WE REALLY ARE VERY FOCUSED ON TRYING TO DO THE VERY BEST SCIENCE AND TRYING TO CREATE THE VERY BEST UNDERSTANDING. SO WHEN WE GO INTO THESE, WE'RE TRYING TO MITIGATE THE SCIENCE RISKS, THAT WE REALLY DON'T KNOW WHAT'S GOING ON OR THAT IT MAY NOT WORK IN THE WAY THAT WE INTENDED. WE'RE ALWAYS GOING TO HAVE THOSE SORTS OF PROBLEMS, BUT WE TRY TO MITIGATE THAT. AND PERHAPS WE'RE A LITTLE MORE SUCCESSFUL THAN SOME OTHER ORGANIZATIONS AROUND THAT.

AND THEN THE OTHER IS IN THE AREA OF CLINICAL

DEVELOPMENT, WHERE, AGAIN, WE SPEND A TREMENDOUS AMOUNT OF TIME TRYING REALLY TO DESIGN STUDIES TO PROVIDE FOR OUTCOMES THAT WE BELIEVE WE CAN ACHIEVE AT THE END OF THE DAY. AND THIS IS A WHOLE DISCUSSION IN AND OF ITSELF, BUT THERE'S A LOT OF DIFFERENT WAYS OF DESIGNING FOR PARTICULAR OUTCOMES. IF, FOR EXAMPLE, YOUR GOAL IS TO HIT A HOME RUN WITH A PRODUCT, TO GO FOR A REALLY LARGE MARKET, THAT COULD BE A MUCH RISKIER PROPOSITION THAN TAKING A MUCH SMALLER SEGMENT OF THE POPULATION WHERE IT'S MUCH CLEARER THAT THE PRODUCT MAY WORK IN AND FOCUSING ON THAT.

ANYWAY, THERE ARE OTHER FACTORS INVOLVED, BUT I THINK THOSE ARE A COUPLE OF KEY ONES FOR US. BUT I WOULDN'T TRY TO BUILD ANY OF THIS INTO ANY RELATIONSHIP YOU HAVE WITH A COMPANY. I THINK IT MORE GOES TO WHO IT IS THAT YOU DECIDE TO FUND, THE LEVEL OF RISK THAT YOU WANT TO TAKE, WHAT THE CRITERIA ARE THAT YOU ARE GOING TO ENGAGE IN TERMS OF EVALUATING WHAT ORGANIZATIONS TO SPEND MONEY WITH OR NOT.

CHAIRMAN PENHOET: OKAY. ANY QUESTIONS FROM IRVINE? FROM L.A.? IF NOT, THANK YOU. I'M SORRY. QUESTIONS FROM THE AUDIENCE?

MR. SIMPSON: JOHN SIMPSON FROM THE FOUNDATION FOR TAXPAYER AND CONSUMER RIGHTS. THIS RELATES -- I'M WONDERING IF YOU IN COMMERCIAL BIOTECH THINK THAT THERE

WOULD BE AN ADVANTAGE IF UPSTREAM PATENTS, MANY OF THOSE THAT ARE DONE BY UNIVERSITIES, WERE IN A PATENT POOL, WHETHER THAT WOULD PERHAPS MAKE LIFE EASIER FOR YOU AND OTHER FIRMS SO YOU COULD GO IN AND KIND OF DO ONE-STOP SHOPPING ON PATENTS THAT COULD BE USEFUL, AND WHETHER IT WOULD PERHAPS BE USEFUL FOR CIRM TO PUT THAT KIND OF RESEARCH AND THOSE KINDS OF PATENTS IN A POOL.

DR. JUELSGAARD: SURE. AS I THINK I RELATED EARLIER, ACTUALLY WHEN IT COMES TO THE NUMBER OF UNIVERSITY PATENTS THAT WE'VE LICENSED, THERE ARE PROBABLY MANY FEWER THAT EXIST THAN EXIST IN THE PRIVATE COMPANY WORLD BECAUSE SO MANY OF OUR PATENTS REALLY RELATE TO THE MOLECULES THEMSELVES, THE USES, ETC., WHICH MOST OF THAT WORK, A LOT OF THAT WORK IS BEING DONE IN COMPANIES. THIS ISN'T REALLY A FUNDAMENTAL ISSUE FOR US. I DON'T -- WE HAVEN'T SEEN SORT OF GROUPS OF PATENTS THAT WE THINK THAT WE NEED TO LICENSE, AND IT'S WORKED RELATIVELY WELL DEALING WITH EACH ORGANIZATION BY ITSELF.

SO THERE MIGHT BE SOME EFFICIENCIES TO BE GAINED, BUT GENERALLY SPEAKING, I DON'T SEE IT AS A PRESSING NEED. BUT THE OTHER IS THAT AT THE END OF THE DAY, AS I UNDERSTAND WHAT CIRM IS GOING TO BE DOING, CIRM HAS A LOT OF MONEY, AND IT'S GOING TO BE PROVIDING THAT MONEY TO ORGANIZATIONS TO SPEND TO DO THINGS. IT MAY BE THAT WHAT CIRM WANTS BACK OUT OF THAT ARE RIGHT TO DEAL

WITH INTELLECTUAL PROPERTY RIGHTS, EITHER TO OWN IT OR TO LICENSE IT OR WHATEVER. THAT'S A FAIRLY UNUSUAL, IN MY EXPERIENCE, A FAIRLY UNUSUAL STRUCTURE FOR THE FUNDING AGENCY, EXCEPT IN THE MARCH-IN RIGHTS SETTING, FOR THE FUNDING AGENCY TO GET BACK, IF YOU WILL, THE INTELLECTUAL PROPERTY RIGHTS AND TO BEGIN TO WORK WITH THEM IN TERMS OF LICENSING THEM TO OTHERS.

TYPICALLY, AS I THINK YOU HEARD FROM THE NIH AND FROM THE JUVENILE DIABETES FOUNDATION, THE ORGANIZATIONS THAT HOLD THE PATENTS WIND UP DOING THE LICENSING, AND ONLY IN EXTRAORDINARY CIRCUMSTANCES DO THOSE ORGANIZATIONS GET INVOLVED. SO I THINK IT WOULD BE FRAUGHT WITH A LOT OF DIFFICULTY, THE NOTION THAT CIRM WOULD SOMEHOW COME BACK AND WANT TO GATHER AND CONTROL IN SOME FASHION PATENT RIGHTS. I JUST FIND THAT A DIFFICULT CONCEPT FOR A LOT OF PEOPLE TO WORK WITH.

CHAIRMAN PENHOET: ANY OTHER COMMENTS? DR. MILMAN.

DR. MILMAN: AS SENATOR DIRKSEN SAID, A BILLION HERE, A BILLION THERE, PRETTY SOON YOU HAVE REAL MONEY. THAT WAS A LONG TIME AGO. AND I THINK, ALTHOUGH THE BILLIONS OF DOLLARS THAT CIRM HAS SEEMS LIKE A LOT OF MONEY, IT REALLY ISN'T, AS YOU JUST INDICATED, IF IT'S GOING TO COST CLOSE TO A BILLION DOLLARS TO PRODUCE.

DR. JUELSGAARD: ABOUT FOUR TO FIVE PRODUCTS IF

YOU LOOK AT IT THAT WAY.

DR. MILMAN: ONE THING I WAS STRUCK BY GENENTECH IS ACTUALLY THE NUMBER OF PRODUCTS THEY'VE DEVELOPED, AND HOW MANY BILLIONS DO YOU SPEND A YEAR?

DR. JUELSGAARD: RIGHT NOW JUST IN RESEARCH AND DEVELOPMENT ABOUT 1.5 BILLION A YEAR.

DR. MILMAN: SO IT'S NOT A LARGE NUMBER. THE NATIONAL INSTITUTE OF ALLERGY AND INFECTIOUS DISEASES IS A $4 BILLION ORGANIZATION A YEAR. THAT'S WHAT WE SPEND. AND IF WE WERE TO SPEND IT ON DEVELOPING DRUGS, WHAT COULD WE DEVELOP? A COUPLE DRUGS A YEAR? WE DON'T DO THAT. SO THE POINT I WANT TO MAKE IS THAT BY PUTTING MONEY INTO SMALL BUSINESSES, AGAIN, I'M THE PROPONENT FOR IT, AND WE ONLY PUT IN $100 MILLION A YEAR, IT'S SORT OF LIKE PLANTING SEEDS FOR A THOUSAND FLOWERS, SOME OF THEM BLOOM. WE ADD ENOUGH VALUES TO THE GENENTECHS, WE'LL GO AHEAD AND BUY THEM. I THINK THAT CIRM IS IN THE SAME POSITION.

CHAIRMAN PENHOET: OKAY. ANY OTHER COMMENTS?

MR. SHEEHY: ONE THING. VERSUS YOUR ACCESS, IS THAT NOT THE INDUSTRY STANDARD NOW, THAT VIRTUALLY EVERYONE HAS AN ACCESS PROGRAM FOR UNINSURED?

DR. JUELSGAARD: YES. THAT WAS MY INITIAL POINT ON THE SLIDE IS THAT THIS IS VERY MUCH THE STANDARD IN THE INDUSTRY. IT VARIES A LITTLE BIT IN TERMS OF THE

LEVELS TO WHICH IT GOES, HOW MUCH SUPPORT IS PROVIDED. SO THERE'S VARIABILITY THERE. YES, GENERALLY SPEAKING, THIS IS THE NORM. AND SO, IN ESSENCE, REQUIRING -- IF ACCESS IS AN ISSUE, THEN HAVING SOME REQUIREMENTS ALONG THESE LINES, I THINK, PERFECTLY LINES UP WITH WHAT REALLY IS GOING ON.

MR. SHEEHY: GREAT. THANK YOU. I JUST WANTED TO REINFORCE THAT.

(APPLAUSE.)

CHAIRMAN PENHOET: I DON'T KNOW ABOUT ANYBODY ELSE IN THE ROOM, BUT I KNOW ABOUT MYSELF. I NEED A FIVE-MINUTE BIO BREAK.

(A RECESS WAS TAKEN.)

CHAIRMAN PENHOET: WE'RE FORTUNATE TO HAVE TWO REPRESENTATIVES FROM PERLEGEN JOIN US THIS MORNING, BRAD MARGUS, THE CEO OF THE COMPANY STANDING BEHIND ME, AND HIS COLLEAGUE, PAUL CUSENZA, SITTING OVER HERE. PAUL IS THE SENIOR VICE PRESIDENT OF MARKETING AND PUBLIC SECTOR COLLABORATION. I DIDN'T EVEN KNOW THERE WAS SUCH A TITLE. WHAT COULD BE MORE RELEVANT TO OUR DISCUSSION TODAY? I THINK, BRAD, YOU ARE GOING TO MAKE THE PRESENTATION. WE'RE DELIGHTED TO HEAR FROM YOU. THANK YOU FOR HELPING US.

MR. MARGUS: THANK YOU. I DON'T KNOW IF I'M GOING TO ADD TOO MUCH TODAY, BUT PERHAPS SOME COLORED

COMMENTARY THAT WILL BE HELPFUL. AS YOU HEARD, PAUL CUSENZA IS IN THE AUDIENCE WITH ME TOO FROM PERLEGEN, AND WE'RE BOTH HERE MORE IN A PERSONAL CONTEXT TOO IN THAT, I THINK, AS YOU WILL HEAR, OUR COMPANY ISN'T REALLY DOING ANYTHING IN STEM CELLS. BOTH THE COMPANY'S EXPERIENCE WITH GOVERNMENT FUNDING AND MY PERSONAL EXPERIENCE PUSHING FOR ADVOCACY ON RARE DISEASE, I MAY HAVE SOME INPUT.

IN SHORT, WHAT PERLEGEN DOES IS WE FOCUS ON USING GENETIC, THE SPECIAL GENETIC CAPABILITY WE HAVE TO TARGET MEDICINES TO THE RIGHT PATIENTS BY FINDING GENETIC MARKERS THAT ARE PREDICTIVE. AND THIS REALLY DOESN'T INVOLVE THE BIOLOGY THAT YOU HEARD ABOUT TODAY ALONG PART OF THAT SPOT, BUT INSTEAD JUST FINDING MARKERS ACROSS THE WHOLE GENOME THAT MAY BE ABLE TO PREDICT WHO SHOULD TAKE A DRUG AND WHO SHOULDN'T.

THIS IS THE ONLY SLIDE WE HAVE ON OUR BACKGROUND, BUT WE WERE FORMED IN EARLY 2001 AS A SPIN-OFF FROM AFFYMETRIX WHERE WE HAD SPECIAL ACCESS TO PLAY WITH THEIR TECHNOLOGY AND DO SOMETHING WITH IT. IN MARCH 2001 WE RAISED A $100 MILLION. WE WENT ON TO DEVELOP OR DISCOVER A LOT OF GENETIC VARIATION USING THE DRAFT OF THE HUMAN GENOME PROJECT AND THEN RESEQUENCING USING 50 MORE GENOMES TO DEVELOP THE CAPABILITY TO REALLY ANALYZE DNA AT A REALLY HIGH RESOLUTION AND A HIGH

THROUGHPUT. RAISED MORE MONEY IN 2003. WE'VE DONE A LOT OF GENETIC ANALYSIS. IT'S CALLED GENOTYPING MORE THAN THE REST OF THE WORLD COMBINED. WE'VE PUBLISHED IN *SCIENCE* NUMEROUS TIMES, A LOT OF OTHER PUBLICATIONS. WE'VE RAISED IN TOTAL ABOUT $250 MILLION IN THE LAST FIVE YEARS, THE LAST TRANCHE COMING LAST DECEMBER WHEN WE RECEIVED $50 MILLION FROM PHIZER, WHO WANTED TO BUY 12 PERCENT OF OUR COMPANY.

ALONG THE WAY WE'VE PARTICIPATED IN SOME VERY BIG PUBLIC CONSORTIUM PROJECTS ON THE INTERNATIONAL HAP MAP PROJECT IS A GOOD EXAMPLE WHERE I THINK WE DID ABOUT 70 PERCENT OF THE GENOTYPING WORK, THE WORK THAT WAS DONE ON A SIX-COUNTRY COLLABORATION. WE'VE ALSO COLLABORATED WITH A LOT OF GOVERNMENTS AROUND THE WORLD.

BASICALLY WHERE WE ARE AS A COMPANY IS WE'VE DEVELOPED A CERTAIN CAPABILITY. WE NOW WORK WITH MOST OF THE TOP PHARMACEUTICAL COMPANIES IN THE WORLD. WE EVEN HAVE A SMALL PILOT WE RECENTLY DID WITH GENENTECH. BEYOND THAT, WE'RE ALSO APPLYING THIS GENETIC CAPABILITY TO OUR PARTNERS' PORTFOLIOS AND OUR OWN LICENSED COMPOUNDS. WE NOW HAVE A PHASE III READY FOR A TYPE 2 DIABETES DRUG. WE HAVE MORE COMPOUNDS ABOUT TO BE LICENSED IN.

BUT ONE THING I WANT TO POINT OUT ABOUT OUR BUSINESS IS THAT IT TURNS OUT THAT OUR CAPABILITY, OUR

GENETIC CAPABILITY, IS BEST APPLIED IN OUR PERSPECTIVE IN COMMERCIAL VALUE, BUT FOCUSING ON THE GENETICS OF DRUG RESPONSE, HOW PEOPLE RESPOND TO DRUGS. YOU TAKE A THOUSAND PEOPLE WHO RESPONDED WELL TO A DRUG AND A THOUSAND PEOPLE WHO'VE HAD AN ADVERSE EFFECT, AND YOU HOPEFULLY FIND MARKERS SO YOU CAN SCREEN PATIENTS.

AT THE SAME TIME THIS CAPABILITY IS ALSO VERY ATTRACTIVE FOR UNDERSTANDING THE GENETIC BASIS OF COMMON DISEASES. AS WE WERE DEVELOPING THIS CAPABILITY, WE WERE CONTACTED BY A LOT OF ACADEMIC AND GOVERNMENT RESEARCHERS AROUND THE WORLD HAD LARGE SAMPLE SETS AND WANTED TO FINALLY GET MORE INFORMATION ABOUT THE GENETIC CAUSES OF ALZHEIMER'S OR PARKINSON'S OR DIABETES OR METABOLIC DISEASE.

SO THIS IS AN IMPORTANT POINT BECAUSE IT WASN'T REALLY CORE TO OUR BUSINESS MODEL NECESSARILY TO GO ABOUT FINDING THESE DISEASE CAUSING GENES WHICH ARE ESSENTIALLY JUST ELUCIDATING NEW PATHWAYS, BUT THEN YOU HAVE TO FIND A DRUG THAT WILL TARGET AND SPEND 15 YEARS OR WHATEVER YOU SAW OUT THERE TILL YOU'RE ACTUALLY AT A THERAPY. BUT IF IT WAS SOMETHING THAT WAS COMPELLING SCIENTIFICALLY TO DO, WE WERE WILLING TO DO IT IF WE COULD FIND OTHER FUNDING FOR IT.

SO OVER THE LAST THREE YEARS, TOOK US THE FIRST TWO YEARS TO REALLY BUILD THE CAPABILITY TO DO WHAT WE

DO. LAST THREE YEARS WE'VE REALLY FORMED A LOT OF COLLABORATIONS. THIS IS A HANDFUL. THERE ARE MANY MORE. BUT, FOR EXAMPLE, MICHAEL J. FOX FOUNDATION, WE DID A COLLABORATION WITH PARKINSON'S DISEASE. THOSE OF YOU ON THE PHONE I'M ON SLIDE 5. AND WE'VE WORKED WITH TEN -- PAUL, YOU CAN CORRECT ME IF ANY OF THESE FACTS ARE WRONG -- BUT TEN DIFFERENT NIH INSTITUTES HAVE FUNDED US. IN SOME SITUATIONS WE MAY COLLABORATE WITH AN ACADEMIC INVESTIGATOR WHO HAS A GRANT OR CONTRACT ALREADY, AND WE BECOME A SUBCONTRACTOR, SO TO SPEAK. IN SOME CASES WE'VE ACTUALLY PROVIDED COST SHARING OR COFUNDED AS A COMPANY AS WE DID SEE SOME VALUE IN DOING THIS, NOT NECESSARILY LONG-TERM IP VALUE ALL THE TIME, MAYBE JUST WE GAIN ORGANIZATIONAL LEARNING. IN OTHER CASES WE HAVE ACTUALLY APPLIED FOR GRANTS OR SBIR GRANTS FROM THE NIH, AND TELL YOU ABOUT THAT EXPERIENCE TOO.

MY PURPOSE IN GIVING YOU THIS BACKGROUND IS JUST SO YOU CAN KIND OF SEE WHAT OUR EXPERIENCE HAS BEEN, AND THEN PERHAPS YOU CAN ASK US QUESTIONS ABOUT OUR EXPERIENCES.

ONE LITTLE CAVEAT ABOUT MY BACKGROUND IS THE WAY I GOT INTO SCIENCE AND BIOTECH EVENTUALLY WAS BECAUSE TWO OF MY SONS WERE DIAGNOSED WITH A REALLY BRUTAL GENETIC DISEASE THAT HAD THEM LOOKING AND APPEARING NORMAL AT AGE 2, BUT TODAY THEY'RE 15 AND 17, AND THEY'RE IN MOTORIZED

WHEELCHAIRS. AND THE MEDIAN AGE OF DEATH FROM THE DISEASE IS ABOUT 17. IT'S A DISEASE WHERE PART OF THE CEREBELLUM IS DEGENERATING. I'VE BEEN VERY KEENLY INTERESTED IN STEM CELLS ALL ALONG. WE'RE A NONPROFIT ORGANIZATION. I STARTED A-T CHILDREN'S PROJECT. WE'VE BEEN FUNDING ABOUT $2 MILLION A YEAR IN RESEARCH, WHICH IS REALLY A DROP IN THE BUCKET, BUT FROM THAT PERHAPS I'VE HAD SOME EXPERIENCE WITH THE GRANT GIVING PROCESS ON THE NONPROFIT SIDE. THE DISEASE IS REALLY RARE. ABOUT 400 KIDS IN THE WHOLE UNITED STATES HAVE IT. ALONG THE WAY I'VE ALSO GOTTEN TO BE AN ADVOCATE FOR A LOT OF DISEASE ORGANIZATIONS AND SAT ON NIH COUNCILS AND SO FORTH.

LOOKING AT THIS, I TRIED TO THINK COMING TODAY TO THINK ABOUT WHAT DO YOU GUYS NEED TO HEAR THAT YOU HAVEN'T HEARD? AND YOU HEARD THE REALLY BIGGER, MUCH MORE SUCCESSFUL MATURE COMPANIES' PERSPECTIVE. I THOUGHT I WOULD JUST TRY TO THINK WHAT DO YOU NEED TO HEAR ABOUT WHAT A SMALLER COMPANY THINKS OF TOO. THE FIRST THING YOU HAVE TO ASK IS I DON'T THINK YOU'RE SITTING IN A POSITION WHERE YOU CAN BE SO CONFIDENT THAT ALL GREAT USERS OF YOUR MONEY AND ACCELERATORS OF STEM CELL RESEARCH WILL COME ON THEIR KNEES TO VISIT YOU. I THINK THAT YOU REALLY SHOULD SEE YOUR SITUATION AS I WOULD DO WITH MY LITTLE NONPROFIT ORGANIZATION AS ONE WHERE YOU

WANT TO USE THIS, IT'S ACTUALLY NOT THAT MUCH MONEY, $3 BILLION OVER TEN YEARS, TO MAKE THINGS HAPPEN THAT OTHERWISE WOULDN'T HAPPEN.

AND HOW ARE YOU GOING TO DO THAT? FOR STARTERS, YOU'D LIKE TO HAVE FIRST-ROUND DRAFT PICKS DOING IT, NOT PEOPLE WHO ARE DESPERATE FOR IT. TO LOOK AT WHO WOULD COME TO THIS CIRM FOR THE FUNDING, I THINK PEOPLE WHO HAVE HIGH RISK RESEARCH WITH UNCERTAIN LIKELIHOOD OF SUCCESS, PEOPLE WHO CAN'T GET FUNDING. YOU HEAR THE SBIR PROGRAM PEOPLE SAY THAT IT SHOULD BE TARGETED WITH PEOPLE WHO DON'T HAVE MUCH VENTURE CAPITAL BACKING AND SO ON. JUST TYPICALLY, ONE ACADEMIC WHO'S GOT AN IDEA AND IS JUST LEAVING OR DOING IT ON THE SIDE WHILE HE'S AT AN ACADEMIC INSTITUTION. YOU MIGHT WANT TO ASK IF THERE ARE LITTLE COMPANIES LIKE THAT THAT CAN'T GET VENTURE CAPITAL FUNDING COMING FOR AN SBIR GRANT, YOU MAY WANT TO ASK WHY CAN'T THEY GET VENTURE CAPITAL FUNDING? MOST BUSINESS IDEAS AROUND HERE, I'M APPROACHED PROBABLY WEEKLY THESE DAYS, HAVING RAISED A LOT OF MONEY, BUT PEOPLE WHO HAVE IDEAS FOR BUSINESSES. AND AGAIN, THE QUESTION IS IF THEY REALLY CAN'T GET ANY EVEN SEED MONEY FROM ANGEL INVESTORS AND ALL THAT, THEIR LAST RESORT IS SBIR. THE STORY YOU MAY HEAR IS IT'S TOO CUTTING EDGE AND TOO VISIONARY, BUT THAT MAY NOT ALWAYS BE THE CASE.

THE OTHER REASON FOR SOME OF MY COMMENTS IS IF

THERE ARE OTHER RESOURCES THEY PREFER OVER YOU AREN'T AVAILABLE, SO BESIDES INVESTORS OF THIS COMPANY, THEY MAY GO TO THE NIH. MOST NIH PROGRAMS WE HAVE, THEY DO HAVE MARCH-IN RIGHTS THAT ARE RARELY IF EVER EXERCISED, BUT IN GENERAL PRETTY MUCH WE DON'T HAVE TO AGREE TO ANY REVENUE SHARING OR IP SHARING OTHER THAN THE CONCERN THAT WE MIGHT NEVER DO ANYTHING WITH IT AT ALL.

IN OUR CASE, TOMORROW IF WHAT MY COMPANY DOES WERE SOMEHOW RELEVANT TO STEM CELLS, I COULD THINK OF A LOT OF IDEAS, BUT I CAN'T THINK OF ANY THAT HAVE GREAT BUSINESS CASE OR COMMERCIALIZATION STRATEGY, THEN PERHAPS SEEING A REALLY COMPELLING SCIENTIFIC ARGUMENT AND HAVING AN ACADEMIC INVESTIGATOR OR SOMEBODY ALREADY FUNDED BY CIRM SAYING WE SHOULD GO GET FUNDING FROM CIRM, WE MIGHT COME TO CIRM AND SAY LET'S DO THIS. IT'S COMPELLING, IT WOULD HELP THE WHOLE WORLD'S STEM CELL EFFORTS GO FORWARD FASTER, AND THAT MIGHT BE SOMEONE WHO COMES TO YOU.

BUT, AGAIN, IN MOST CASES, IF A COMPANY IS COMING TO YOU, IT'S PROBABLY BECAUSE THEY'RE HAVING TROUBLE EITHER INTERNALLY OR WITH INVESTORS CONVINCING PEOPLE THAT THERE'S GOING TO BE A HUGE MARKET POTENTIAL OR A FAST ENOUGH MARKET PATH FOR WHAT THEY WANT TO DO.

THIS OTHER ONE IS A LITTLE BIT OF A SNIDE COMMENT, BUT PAUL AND I AT BREAKFAST THIS MORNING DECIDED TO LEAVE THIS IN, AND THAT IS, TO BE CANDID, A COMPANY IS

NOT ABOUT TO COME TO CIRM OR THE NIH IF THEY REALLY HAVE AN URGENT THING. THIS IS GOING TO BE SOMETHING WHERE IT'S A LITTLE BIT MORE LESS TIME SENSITIVE AND YOU FEEL LIKE YOU CAN MOVE FAST. I SAY THIS BECAUSE FROM MY NONPROFIT HAT'S POINT OF VIEW, ONE OF THE WAYS THAT WE FEEL A NONPROFIT ORGANIZATION CAN SOMETIMES MAKE RESEARCH HAPPEN THAT OTHERWISE WOULDN'T, WHICH IS OUR MAIN OBJECTIVE, IS BY REALLY SELLING TO THE RESEARCH COMMUNITY THAT OUR GRANT REVIEW PROCESS IS FASTER, THAT WE CAN MAKE DECISIONS FASTER. IF YOU DON'T HAVE THAT, THEN AGAIN, GOING BACK TO THAT FIRST THING I SAID, WHICH IS WE WANT TO BE RECRUITING THE BEST AND THE BRIGHTEST TO WORK ON STEM CELLS, THAT'S NOT GOING TO HAPPEN.

AS I SAT THERE TODAY THINKING, WEARING MY FATHER'S HAT WITH TWO KIDS WHO COULD REALLY USE STEM CELLS AS THE ONLY WAY THAT YOU COULD TREAT A DISEASE LIKE MY KIDS' DISEASE TODAY, AND THEN I HEARD GENENTECH'S PRESENTATION, THE FIRST THING ON MY MIND WAS IT'S A FANTASY, BUT WHAT WOULD IT TAKE TO GET GENENTECH TO PUT ALL $1.5 BILLION A YEAR IN STEM CELLS? HOW DO YOU GET THEM TO DO THAT? NOT HOW DO YOU GET SOMEBODY WHO HAS A BUSINESS IDEA TO COME ALONG AND DO IT, NOT THE PRO'S WHO KNOW WHAT THEY'RE DOING.

I MENTIONED ANOTHER WAY THAT YOU CAN END UP IN PERHAPS A COMPANY WORKING WITH CIRM WOULD BE IF OUR

COLLABORATORS ON SOMETHING ELSE ARE ALSO WORKING WITH CIRM.

THEN THERE'S THIS WHOLE THING WHICH IS MAYBE A LESSON THAT CAN BE LEARNED FROM THE GENETIC SPACE. I MENTIONED THAT WITH FINDING THE GENES FOR DISEASES, COMPANIES LIKE INSIGHT AND CELERA AND A BUNCH OF OTHER ONES IN THE GENOME SCIENCES IN THE LATE '90S FOR PATENTING EVERY GENE IN SIGHT WHETHER IT WAS KNOWN OR PREDICTED IN THE GENOME WITHOUT FUNCTIONING. IN THOSE DAYS THERE WAS A LAND GRAB GOING ON. TODAY, EVEN IF YOU FIND THE GENES ASSOCIATED WITH THE DISEASE, A LOT OF PHARMACEUTICAL COMPANIES WILL TELL YOU THEY DON'T REALLY CONSIDER THAT IP OR PROTECTABLE TYPE INFORMATION ANYMORE. THEY NOW CONSIDER IT IN A CATEGORY THAT THEY CALL PRECOMPETITIVE. AND SO A LOT OF THE RESEARCH PERHAPS THAT WE'RE DOING RIGHT NOW IN STEM CELLS WHERE THE STATE OF THE SCIENCE IS IS REALLY THAT PRECOMPETITIVE STUFF ANYWAY. AND MAYBE EVEN THE WAYS PEOPLE ARE THINKING ABOUT PROTECTING IT RIGHT NOW WE'LL EVENTUALLY ALL AGREE FIVE YEARS FROM NOW THAT THAT STUFF IS PRECOMPETITIVE.

IF SOMEONE CAN LEARN SOONER THAN ANYBODY ELSE WHICH GENES CONTROL DIFFERENTIATION OR MIGRATION OF STEM CELLS, THAT'S KIND OF COMMON KNOWLEDGE. THE BIG DRUG DEVELOPERS IN THE WORLD WILL CONSIDER IT PRECOMPETITIVE, AND THEY ALL BELIEVE THAT THEY'RE THE FASTEST AT TAKING

THOSE DISCOVERIES AND THEN MAKING IT INTO SOMETHING TRULY PROPRIETARY. SO THEY'RE NOT TOO WORRIED ABOUT PROTECTING IT. AND IT'S IMPORTANT TO THINK FOR THE ACTUAL THING, AS WE'RE SETTING UP ALL THESE STRUCTURES AND THINKING ABOUT IP, HOW MUCH THAT'S GOING TO BE DISCOVERED IN THE NEXT FIVE YEARS IS GOING TO BE REALLY PROTECTABLE.

WHAT ARE OUR CONCERNS? OBVIOUSLY THE DISCLOSURE IP REVENUE SHARING THAT YOU HEARD ABOUT IS A CONCERN. AS I READ THROUGH YOUR POLICY FOR THE NONPROFIT IP, I THOUGHT THAT ONE THING THAT WAS IMPORTANT TO KEEP IN MIND WAS WHAT WE CALL PLATFORM INVENTIONS. SO IF A COMPANY WERE FUNDED BY CIRM AND DISCOVERED SOME NEW INFORMATION ABOUT STEM CELLS THAT COULD HELP THE WORLD, MAYBE YOU WANT TO HAVE IT AVAILABLE TO THE WORLD ON SOME TIME FRAME BECAUSE YOU FUNDED IT. AT THE SAME TIME THAT THEY'RE DOING THAT, IF WE DEVELOP OR IF WE FIND A WAY TO IMPROVE OUR OWN TECHNOLOGY THAT'S UNIQUE TO OUR COMPANY, WE WOULDN'T WANT TO GIVE THAT TO THE WORLD. AND THE WAY THE DOCUMENT I READ QUICKLY APPEARED TO ME WAS THAT IT DIDN'T REALLY GET VERY SPECIFIC ABOUT WHAT KIND OF INVENTIONS WERE MADE. I THINK COMPANIES WILL BE VERY SENSITIVE IF THEY HAVE A UNIQUE PLATFORM AND YOU'RE GOING TO NOW HAVE SOME RIGHTS TO THAT TOO.

SHARING OF MATERIALS, IF YOU'RE MAKING KNOCKOUT MICE, NO ACADEMIC RESEARCHER THAT MAKES KNOCKOUT MICE

AFTER THEY PUBLISH IT IS THRILLED ABOUT BECOMING A MOUSE SUPPLY HOUSE FOR THE WHOLE WORLD, ESPECIALLY IF IT'S A REALLY INTERESTING MOUSE, BUT THAT'S SOMETHING THEY DEAL WITH AND THEY GET OVER AND HOPEFULLY FIND FUNDING AND HAVE SOMEONE LIKE JACKSON LAB DO IT. BUT IN THE CASE OF SOME THINGS COMPANIES DO, IT MAY BE MUCH MORE COSTLY. IN OUR CASE WE USE SOME ARRAYS THAT COST, IF YOU DROP ONE, IT'S AS MUCH AS DROPPING A LEXUS. TO REPLICATE AN EXPERIMENT YOU MAY PUBLISH MAY BE ACTUALLY MORE EXPENSIVE IF YOU'RE GOING TO MAKE THOSE REAGENTS AVAILABLE TO EVERYONE.

IF THE LIABILITY FOR THE REVENUE SHARING THAT IS PROPOSED IS NOT CAPPED, THAT IS A SERIOUS ISSUE IF YOU TALK TO ANY BIOTECH CEO WHO HAS TO DEAL WITH HIS INVESTORS. THAT'S A REAL CHALLENGE.

THE OTHER THING TO KEEP IN MIND, WHICH YOU HEARD A LITTLE BIT, YOU HEARD ABOUT THE LONG DEVELOPMENT TIME. THE OTHER THING TO KEEP IN MIND IS THE PATENT LIFE AFTER THAT. AND THAT PATENT LIFE IS LIMITED. IN THE SCHEME OF THINGS FOR CIRM, YOU GOT TO REMEMBER THAT ONCE THEY FINALLY GET IT TO MARKET, EVEN IF IT'S A GREEDY, HOARDING, BIG PHARMA THAT'S DOING THIS, THEIR TIME RUNS OUT REALLY, REALLY FAST. IT'S THE NO. 1 TOPIC AT EVERY PHARMA CONFERENCE THESE DAYS IS THEIR PIPELINES ARE EXPIRING TOO QUICKLY. IT WILL BE MADE AVAILABLE TO THE

WORLD SOONER THAN YOU THINK.

ALONG THOSE LINES, THE OTHER TOPIC THAT YOU MIGHT WANT TO THINK ABOUT TOO IS, I DON'T KNOW HOW YOU'RE DEALING WITH IT, BUT THESE INVENTIONS, INEVITABLY THERE ARE STRATEGIES TO EXTEND THEIR PROTECTION. I DON'T KNOW HOW YOUR POLICY IS GOING TO CONTINUE TO GET A PIECE OF THAT ACTION TOO, OR WILL YOU ONLY HAVE A PIECE OF THE ACTION ON THE ORIGINAL PATENT.

FOR MY LAST BASIC COMMENTS I'LL MAKE, WEARING TWO HATS, IN TURNING TO THE SUBJECT THAT WHAT SHOULD CIRM CARE ABOUT, I UNDERSTAND THAT THERE ARE PROBABLY MANY DIFFERENT CONSTITUENTS SUPPORTING CIRM THAT VOTED ON AND MADE THE PROPOSITION PASS, AND YOU'VE GOT TO SATISFY ALL OF THEM. IT WASN'T CLEAR TO ME WHEN I READ IN THE NEWSPAPER THAT IT WAS PASSED THAT CIRM HAD SOME NEED TO BE IN SOME WAYS VENTURE CAPITALISTS OR SOMETHING THAT GETS SOME RETURN ON THE $3 BILLION IN TERMS OF FINANCIAL RETURN. IT WASN'T CLEAR TO ME THAT, ALTHOUGH ACCESS IS ABSOLUTELY IMPORTANT, IT WASN'T CLEAR TO ME THAT CIRM WAS GOING TO NEED TO HELP MAKE UP FOR WHERE INSURANCE ISN'T COVERING PEOPLE.

BUT WHEN YOU ASK WHAT IS THE PRIMARY PURPOSE OF THIS SHOT IN THE ARM OF THE $3 BILLION, OTHER THAN WHAT IT DOES FOR CALIFORNIA AND ALL THAT, I WOULD HOPE THAT IT'S TO MAKE SOMETHING HAPPEN THAT OTHERWISE WOULDN'T

HAPPEN. ALONG THOSE LINES, HAPPENS FASTER THAN IT WOULD HAVE HAPPENED. AND THEN THE POINT I MADE AT THE VERY BEGINNING IN MY COMMENT ABOUT GENENTECH, I THINK YOU WANT TO ENCOURAGE THE BEST-IN-CLASS PARTICIPATORS TO PARTICIPATE, NOT JUST THE NEEDY ONES. WHEN IT COMES TO YOUR OWN KIDS, IT'S PERFECTLY FINE IF THE ONE KID ISN'T QUITE THAT SHARP, YOU WANT TO HELP HIM OUT, EQUALIZE THINGS, THAT'S FINE. BUT WHEN WE'RE TALKING ABOUT THIS LIFE OR DEATH MATTER AND REALLY MAKING A DIFFERENCE, I THINK YOU SHOULD ONLY GO WITH THE COMPANIES THAT CAN MAKE IT HAPPEN THE FASTEST.

I'LL TELL YOU ONE OTHER COMMENT I THINK I MADE TO MARY WHEN SHE CALLED ME ON THE PHONE. THAT WAS A REALLY FRUSTRATING THING WITH ME WITH THE NIH IS THAT IF THERE WERE THIS IMPORTANT ENDEAVOR NEEDED, I DON'T KNOW WHAT THAT WOULD BE, BUT SOME NEW KIND OF STEM CELL LINES THAT HAD TO BE MADE VERY RAPIDLY, THOUSAND STEM CELL LINES YOU WANTED TO MAKE, AND THE NIH PUT OUT AN RFA FOR IT TO COMPETE SO IT DOESN'T LOOK CONFLICTED OR ANYTHING, YOU COMPETE IT REALLY WELL. AND TEN DIFFERENT ACADEMIC INSTITUTIONS INCLUDING A FEW CONSORTIUMS THAT SOUND GREAT, NOBEL LAUREATES BEHIND THEM, ALL SUBMIT THEIR PROPOSALS AND THEY'RE ALL AROUND THE $50 MILLION RANGE FOR WHAT THEY WANT TO DO. AND A COMPANY COMES ALONG THAT CAN DO IT FOR $2 MILLION, BUT THEIR INVESTORS HAVE THIS

WEIRD THING ABOUT THEM THAT THEY INSIST THAT THE COMPANY ACTUALLY MAKES A PROFIT. SO THE COMPANY WANTS TO CHARGE 3 MILLION FOR IT.

IN SOME OF THOSE SITUATIONS THAT I JUST DESCRIBED, THE NIH WILL HAVE A POLICY THAT IT HAS TO BE COST RECOVERY OR THAT THE COMPANY IS ALLOWED TO MAKE COSTS AND OVERHEAD AT SOME OVERHEAD RATE OR AT BEST COST AND OVERHEAD AND A 10-PERCENT PROFIT OR SOMETHING. AND SO IN THE END THE GRANT WILL GO TO THE $50 MILLION OR $49 MILLION SUBMITTER BECAUSE THEY CAME IN THE LOWEST AND WERE WILLING TO LIVE WITH THOSE TERMS. MEANWHILE IT WOULD HAVE BEEN A LOT SMARTER FOR THE NIH OR FOR CIRM OR FOR OUR GOVERNMENT TO FUND THE $3 MILLION ONE AND LET THE PEOPLE MAKE THE WHOPPING 50-PERCENT MARGIN.

I'D LIKE CALIFORNIA TO TRY TO DO IT DIFFERENTLY THAN THE GOVERNMENT IF IT CAN AND THINK A LITTLE SMARTER. IF THERE REALLY IS A COMPANY OUT THERE THAT HAS THE NEXT THING THAT YOU NEED IN STEM CELL RESEARCH TO HAPPEN AND YOUR OBJECTIVE SCIENTIFIC ADVISORS SAY THIS IS WHAT WE WANT, YOU SHOULD BE REALLY, REALLY AGGRESSIVE IN GETTING IT. AND I'M CONCERNED THAT SOME OF THESE THINGS THAT WE'RE THROWING OUT THERE, LIKE REVENUE SHARING OR THE CAPPING WHAT A COMPANY, WILL ONLY DEFEAT THAT PURPOSE.

I KNOW THIS IS A PROBLEM. THE SBIR GUY IS RIGHT. ONCE YOU HAVE A BIG INVENTION AND STARTS MAKING

MONEY AND IF THE CLAUSE WAS THAT IT SOMEHOW IS BASED ON HOW MUCH EACH PARTY PUT IN, THE COMPANY IS PROBABLY GOING TO SCRATCH AND FIND EVERY DOLLAR THEY EVER PUT INTO IT AND TRY TO JUSTIFY IT THAT WAY. ON THE FLIP SIDE OF THAT STORY IS THAT IF YOU ONLY LOOK AT A COMPANY AND WHAT THEY'RE PUTTING INTO A PARTICULAR PROJECT THAT CIRM IS FUNDING AND THEN BASING THE RETURN THAT CIRM GETS OR THEY GET ON THAT INVESTMENT, YOU MAY BE FORGETTING THE TREMENDOUS INVESTMENT THAT WENT ON TO CREATE THAT PLATFORM OR THAT TECHNOLOGY OR THAT INFRASTRUCTURE THAT MAKES THEM THE BEST IN THE WORLD TO DO IT.

(INTERRUPTION IN PROCEEDINGS.)

MR. MARGUS: I'M ON SLIDE 9. I'M ALMOST DONE. NEXT POINT WAS THAT A LOT -- IF YOU LOOK AT THE GRANTS THAT A LOT OF COMPANIES HAVE GOTTEN FOR THE FOR-PROFIT WORLD, YOU SHOULD REALLY LOOK AT HOW MANY OF THEM -- IF THEY'RE SBIR'S, I'D LIKE SOMEONE TO LOOK AT HOW MANY OF THEM ACTUALLY PRODUCED A SUCCESSFUL COMPANY OUT OF THEM. IF IT'S A GOVERNMENT GRANT FOR FUNDED RESEARCH, GIVEN THE REASONS WHY I THINK MOST OF THE TIME COMPANIES EVEN LOOK TO THE GOVERNMENT FOR FUNDING, I THINK YOU'LL SEE THAT A LOT OF TIMES IT'S ALMOST ON THE BORDER OF ALTRUISM, OR AS I DID WITH MY COMPANY, WHERE THERE WAS SOMETHING THAT WAS COMPELLING TO DO AND WE WERE WILLING TO DO IT IF THERE WAS SOMEBODY ELSE WILLING TO FUND IT, EITHER THE

GOVERNMENT OR THE NONPROFIT FOUNDATIONS.

BUT IF YOU REALLY WANT THE BEST COMPANIES TO TAKE STEM CELLS OUT OF THE HANDS OF THE ACADEMICS OR THE INVESTIGATORS THAT DO THE BASIC SCIENCE AND TAKE IT ALL THE WAY TO THE CLINIC, WHICH IS WHAT WE'RE ALL WAITING FOR, AND COULD MAYBE HAPPEN FASTER THAN PEOPLE THINK, THEN YOU'VE GOT TO FIND A WAY TO GET THE PLAYERS WHO ARE FOR-PROFIT COMPANIES INVOLVED. YOU CANNOT IGNORE THEM OR JUST PLAY WITH LITTLE START-UP COMPANIES. AND FOR THAT I HAVE TO SAY THAT YOU HAVE APPEAL TO THEIR GREED. DON'T TRY TO COUNT ON THEM BEING ALTRUISTIC.

AGAIN, ON THE RETURN, I THINK THAT TO HAVE AN UPSIDE WHERE YOU HAVE THAT ROYALTY OR SOMETHING THAT GOES ON OR 25 PERCENT OR WHATEVER GOES ON AND ON AND ON, I THINK, IS UNNECESSARY. EARLIER TODAY WE HEARD SOMEONE SAY SOME MULTIPLE OF THE INVESTMENT YOU MADE TO FIVE X, TEN X TO WHATEVER YOU GAVE THEM IN A GRANT THAT YOU REQUIRED BACK, THAT'S FINE, BUT I JUST THINK TO HAVE THE UNLIMITED UPSIDE WILL BE REALLY HARD FOR THAT COMPANY'S INVESTORS TO TAKE.

MY LAST IS SLIDE, AGAIN, THE WORST-CASE SCENARIO, AS A FATHER OF TWO SONS THAT COULD REALLY BENEFIT FROM STEM CELL RESEARCH, THE QUESTION TO ASK IS YOU SHOULD START WITH JUST A VERY, VERY WORST-CASE SCENARIO. IF CIRM GAVE A GRANT THAT ENDED UP THROUGH A

LOT OF OTHER DEVELOPMENT AFTERWARDS PRODUCING A TREATMENT EVEN A YEAR SOONER AND IT HELPED A LOT OF PEOPLE, WOULD IT BE THE END OF THE WORLD THAT CIRM DIDN'T MAKE A KILLING OFF OF IT? SO THANKS A LOT.

(APPLAUSE.)

MR. MARGUS: I DON'T KNOW IF YOU HAVE ANY QUESTIONS FOR EITHER ME OR PAUL CUSENZA IS WITH ME, OUR SENIOR VICE PRESIDENT OF MARKETING AND PUBLIC COLLABORATIONS, IS REALLY MUCH MORE FAMILIAR WITH THE MECHANISMS THAT WE'VE USED WITH ALL THE DIFFERENT NONPROFIT ORGANIZATIONS AND WITH THE NIH AND SBIR STUFF.

CHAIRMAN PENHOET: ONE QUESTION. YOU GOT A LOT OF COLLABORATION. IN SOME OF THOSE CASES WERE YOU FUNDED BY THE COLLABORATOR OR, IN GENERAL, YOU'VE JUST DONE YOUR PART OF A PROJECT AND THEY'VE DONE THEIR PART OF A PROJECT?

MR. MARGUS: MOST OF THE ONES YOU SAW UP THERE, THEY ACTUALLY FUNDED US.

CHAIRMAN PENHOET: THEY DID PROVIDE FUNDING.

MR. MARGUS: FOR EXAMPLE, MICHAEL J. FOX FOUNDATION GAVE US $3 MILLION TO LOOK AT THE GENETICS OF PARKINSON'S DISEASE.

CHAIRMAN PENHOET: WHAT DID THEY EXPECT IN RETURN?

MR. MARGUS: JUST DISCOVERIES AND A PAPER CAME

OUT OF IT AND THERE WAS PUBLICATION. THEY HAVE A LOT OF EFFORTS GOING ON; BUT IF THEY COULD FIND NEW LEADS TO UNDERSTANDING THE PATHOGENESIS, THE BIOLOGICAL BASIS OF PARKINSON'S, THEY FELT THEIR WHOLE FIELD WOULD BE ACCELERATED. THEY COULD FOCUS THEIR RESEARCH BETTER. BUT IT'S VERY DIFFERENT THAN IF YOU TAKE A COMPOUND THAT WE'VE LICENSED THAT WE'RE WORKING ON NOW TO TAKE THROUGH PHASE III AND LAUNCH, IF MICHAEL J. FOX CAME ALONG AND SAID WE'LL GIVE YOU $3 MILLION AND WE WANT TO MAKE THAT IP ALL FREE TO THE WORLD, THAT WOULD BE TOUGH FOR US TO DO.

DR. KOVACH: SO IN THE MICHAEL J. FOX, WHAT DID THE -- I'M SURE THERE WAS AN IP COMPONENT TO THE CONTRACT. MY NAME IS JIM KOVACH FROM THE BUCK INSTITUTE FOR AGE RESEARCH. I WAS JUST WONDERING HOW THE IP PROVISION READ IN MICHAEL J. FOX AND WHETHER ANY -- IN THE $3 MILLION, WHETHER ANY DISCOVERIES CAME OUT OF THAT WORK?

MR. CUSENZA: IN ALL THESE CASES THEY CAN BE A LITTLE BIT DIFFERENT, AND THE EXACT TERMS OF THAT ARRANGEMENT WERE NOT DISCLOSED, BUT THAT WAS A STUDY WHERE WE APPLIED FOR IT WITH THE MAYO CLINIC, SO ACTUALLY WE PARTNERED UP WITH THE MAYO CLINIC, WHICH IS OFTEN WHAT WE DO. SO THAT THE EXPERTS IN PARKINSON'S DISEASE, THEY TREAT THEM, THEY HAD THE SAMPLES. AND MICHAEL J. FOX WAS

TRYING TO FUND RESEARCH THAT OTHERWISE WOULDN'T GET ACCOMPLISHED OR DONE. IT'S LIKE 70 PEOPLE HAD APPLIED FOR THIS PARTICULAR GRANT, AND THEY EVENTUALLY AWARDED A GRANT TO US. I THINK THE FIRST IN THIS LEAP PROGRAM TO SORT OF MOVE THINGS FORWARD TO GO FASTER. AND ACTUALLY AT A VERY FAST CYCLE IN TERMS OF THE TURNAROUND OF APPLYING TO ACTUALLY AWARDING IT.

IN TERMS OF THE IP, IN THOSE KINDS OF SITUATIONS SOMETIMES THERE IS, IN FACT, A SHARING. AS YOU HEARD FROM THE JDRF EXAMPLE, OFTENTIMES YOU CAN DO, AND I'M GOING TO TALK ABOUT THIS NOT SPECIFICALLY, BUT BROADLY TO THINK ABOUT IT, IS THERE CAN BE SHARING THAT CAN HAPPEN AMONG THE DIFFERENT PARTIES IN AN EQUITABLE WAY, RIGHT? AND THERE'S OTHER ORGANIZATIONS THAT DO SOMETHING LIKE THIS TOO. FOR EXAMPLE, THERE'S THE UK, LIKE THE CANCER RESEARCH UK, AND THEY'VE GOT AN ORGANIZATION, CANCER RESEARCH TECHNOLOGY, WHICH IS THEIR FOR-PROFIT SUBSIDIARY WHICH CAN ALLOW SHARING OF IP AND OTHER DIMENSIONS.

OFTENTIMES IN THESE CASES, AS BRAD WAS ALLUDING, YOU WANT TO MAKE SURE THAT IT'S REASONABLE IN THE SENSE THAT, YES, IF SOMETHING COMES UP AND IT'S A GREAT COMMERCIAL SUCCESS, YOU DON'T WANT THEM SORT OF FEELING LIKE, GOSH, THEY WERE LEFT WITH NOTHING AT ALL. ON THE OTHER HAND, YOU ALSO WANTED TO HAVE IT BE FAIR TO ALL THE PARTIES IN WHAT MAY BE GOING FORWARD BECAUSE IT'S VERY

COMPLEX IN TERMS OF DEFINING WHO SHOULD GET WHAT SHARE, WHAT OTHER INVESTMENT GOES ON. AND AS DESCRIBED BY JDRF, IT'S CASE BY CASE BECAUSE THESE SITUATIONS ARE VERY DIFFICULT TO SOMETIMES WORK OUT. SO IT'S OFTEN HOW THAT WORKS WHEN IT IS WITH THE FOUNDATIONS, WHICH IS A LITTLE BIT DIFFERENT.

OF COURSE, WITH THE NIH, IT'S DIFFERENT SITUATIONS. OFTENTIMES WE COLLABORATE, SAY, WITH ACADEMICS, AND WE THEN PARTNER UP TOGETHER. BUT THEN THE AWARD, WHICH COMES FROM THE NIH, PERMITS THEN THE PARTIES WHO ARE INVOLVED TO SHARE THE IP. OFTENTIMES THEN THERE'S AN ARRANGEMENT THAT THEN IS WORKED OUT BETWEEN US AND THE NONPROFIT INSTITUTION THAT ARE WORKING TOGETHER.

MR. MARGUS: BASICALLY IT'S USUALLY NEGOTIATED CASE BY CASE, BUT THE FUNDING ORGANIZATION IS GOING TO WANT SOMETHING. AND IF YOU'RE WORKING WITH AN ACADEMIC COLLABORATOR ON A DISEASE, THEY'RE GOING TO WANT SOMETHING. WITHOUT A DOUBT THE ACADEMIC INSTITUTION ALWAYS WANTS SOMETHING, BUT IT MAY BE SPLITTING IT THREE WAYS. THE INTELLECTUAL PROPERTY, AGAIN, IF IT LOOKS REALLY, REALLY BASIC RESEARCH THAT'S GOT A LONG ROAD AHEAD, THERE SEEMS TO BE LESS DEBATE THAN IF SOMEONE SEES A DIAGNOSTIC COMING RIGHT OUT OF THE DISCOVERIES.

CHAIRMAN PENHOET: OKAY. ANY QUESTIONS FROM THE TASK FORCE HERE?

DR. FONTANA: I HAVE A QUESTION. JEANNIE FONTANA FROM L.A. BRAD, I REALLY ENJOYED YOUR PRESENTATION. AS A PATIENT ADVOCATE, YOU APPEAL TO MY SENSE OF URGENCY AND EFFICIENCY BY WHICH YOU TRY TO COME UP WITH THERAPIES. I'M CURIOUS, THOUGH, AS YOU TALK ABOUT WHAT CIRM SHOULD BE CONCERNED ABOUT, APPEALING TO COMPANIES' GREED, TRYING TO PULL IN THE NO. 1 DRAFT CHOICE, HOW DO YOU THINK CIRM SHOULD HANDLE THE PUBLIC'S PERCEPTION OF TAXPAYER DOLLARS GOING TO A FOR-PROFIT COMPANY THAT MAY BE THE MOST EFFICIENT WAY OF DEVELOPING A THERAPY, BUT FOR SOME REASON THAT'S PERCEIVED AS GIVING AWAY MONEY, HARD-EARNED TAXPAYER DOLLARS AWAY TO THE GREEDY PHARMACEUTICAL INDUSTRY. HOW WOULD YOU SUGGEST WE APPROACH THAT?

MR. MARGUS: PERCEPTION IS REALLY TOUGH BECAUSE PEOPLE CAN CONSTRUE IT AND TWIST IT TO SOUND LIKE ANOTHER BIG PHARMA IS GOING TO GET RICH OFF OF THE DISCOVERY. IF TOMORROW WE HAD SOMETHING READY FOR CLINIC, I MAKE THAT AS AN IMPORTANT MILESTONE BECAUSE THAT'S WHEN THE DOLLARS REALLY GO UP AND YOU REALLY NEED A LOT OF EXPERTISE THAT ISN'T USUALLY DONE IN ACADEMIC SETTINGS. IF TOMORROW WE HAD A STEM CELL TREATMENT READY FOR THE CLINIC, THERE ARE TWO ROADS YOU CAN GO IF YOU'RE CIRM. ONE WOULD BE TO SOMEHOW HAVE THE INFRASTRUCTURE AT CIRM TO USE CRO'S AND BID THEM OUT AND HAVE THE CRO'S DO IT. IT'S VERY LIKELY

YOU'RE GOING TO HAVE THE EXPERTISE, THE THERAPEUTIC AREA EXPERTISE AND EVERYTHING ELSE TO TAKE THAT WHOLE ROAD. I THINK YOU WANT A PARTY INVOLVED TO PARTNER WITH CIRM THAT'S GOING TO TAKE IT FORWARD THAT KNOWS HOW TO DO THIS IN THEIR SLEEP AND CAN GET IT THERE.

I THINK I COULD CONVEY THAT TO THE PUBLIC, THAT, AGAIN, IF IT'S BEEN CREDIBLE ALL ALONG THAT YOUR OBJECTIVE HERE IS TO MOVE AS QUICKLY AS POSSIBLE, IF THE SELECTION OF THAT PARTNER TO TAKE THE RESEARCH FORWARD, WHATEVER COMPANY IT WAS, WAS A VERY OBJECTIVE PROCESS WITH CLEAR CRITERIA, I DON'T THINK YOU WOULD BE CASTIGATED THAT MUCH.

DR. FONTANA: I WISH THAT WERE THE CASE. IT DOESN'T SEEM TO BE THAT WAY.

MR. MARGUS: I'LL HELP YOU DO IT. JUST GET ME A THERAPY READY FOR CLINICAL TRIALS.

DR. FONTANA: THANK YOU.

CHAIRMAN PENHOET: ANY OTHER QUESTIONS FROM THE NON-SAN FRANCISCO SITES? FROM OUR AUDIENCE HERE IN SAN FRANCISCO? IF NOT, WE'LL THANK BRAD AND PAUL.

(APPLAUSE.)

CHAIRMAN PENHOET: BRAD, YOU ARE A VERY EFFECTIVE SPOKESMAN. SO WHO KNOWS. WE MIGHT TAKE YOU UP ON YOUR OFFER, WHICH YOU MADE IN PUBLIC.

SO OUR FINAL SPEAKER IS JAMES KOVACH. JAMES IS

PRESIDENT AND CEO OF THE BUCK INSTITUTE. AND WE'VE ASKED JAMES -- WELL, TO REVIEW WHERE WE'VE BEEN TODAY, OUR FIRST SPEAKER WAS FROM THE FEDERAL GOVERNMENT, A MAJOR GRANT MAKER TO ALL KINDS OF ORGANIZATIONS, INCLUDING BUSINESS. WE THEN HEARD FROM ROBERT GOLDSTEIN, WHO IS ALSO A FUNDER OF PROJECTS IN THE PRIVATE SECTOR. AND NOW WE'VE HAD TWO TALKS FROM PEOPLE IN THE PRIVATE SECTOR WHO ARE EITHER RECIPIENTS OF FUNDING FROM PUBLIC SOURCES OR POTENTIAL RECIPIENTS. AND NOW WE'VE ASKED JAMES KOVACH TO GIVE US A PERSPECTIVE OF SOMEONE WHOSE CURRENT POSITION IS ACTUALLY LICENSING TECHNOLOGY TO COMPANIES BECAUSE THAT'S IN SOME SENSE RELEVANT TO WHAT WE'LL BE DOING AS A FUNDER BUT ALSO AS A LICENSOR. JAMES.

DR. KOVACH: THANKS A LOT FOR THE OPPORTUNITY TO COMMENT AND TAKE QUESTIONS. WHAT I'D LIKE TO DO IS TO TALK ABOUT THE BUCK INSTITUTE'S PHILOSOPHY AND POLICY. WE ARE THE ONLY INDEPENDENT RESEARCH INSTITUTE IN THE WORLD DEDICATED EXCLUSIVELY TO AGE RESEARCH AND AGE ASSOCIATED DISEASES, AND CERTAINLY THE CONNECTION BETWEEN STEM CELL EXHAUSTION AS PEOPLE AGE AND OUR INSTITUTE IS VERY STRONG. WE PLAN TO PARTICIPATE IN CIRM FUNDING AND WITH COMPANIES AS WELL.

WE'RE ALL BASICALLY OFFSPRINGS OF OUR OWN BACKGROUND, AND SO I JUST WANTED TO MENTION THAT MY BACKGROUND INCLUDES SEVERAL YEARS RUNNING THE

TECHNOLOGY -- CREATING A TECHNOLOGY MANAGEMENT PROGRAM AT CASE WESTERN RESERVE SCHOOL OF MEDICINE. THEN I ACTUALLY SPUN OUT A COUPLE OF COMPANIES AND BECAME EVP OF A COMPANY IN CLEVELAND, OHIO, THAT PARTICIPATED IN THE STEM CELL ARENA. AND I GOT VERY INTERESTED IN LAW SCHOOL ABOUT THE INTERFACE BETWEEN ACADEMIC MEDICINE AND BUSINESS. SO I WAS A VERY STRONG PARTICIPANT IN THE BIOTECHNOLOGY INDUSTRY ORGANIZATION. IN FACT, I UNITED WITH MY COLLEAGUES AT AUTM, THE ASSOCIATION OF UNIVERSITY TECHNOLOGY MANAGERS, TO CREATE A SUBCOMMITTEE ON TECHNOLOGY TRANSFER REALLY TO LOOK AT THIS INTERFACE AND HOW TO TRY TO OPTIMIZE IT.

SO WHAT HAS HAPPENED AS TECHNOLOGY TRANSFER IN UNIVERSITIES HAS GOTTEN VERY SOPHISTICATED AFTER PASSAGE OF THE BAYH-DOLE ACT AND MANY OF THE EARLY SUCCESSES, THAT THERE'S A VERY -- IT'S A VERY UNIFORM PRACTICE IN UNIVERSITIES AND ONE THAT THE BUCK INSTITUTE FOLLOWS IN TERMS OF LOOKING, SURVEILLING THE LANDSCAPE OF OUR OWN RESEARCH. I MEAN TYPICALLY UNIVERSITIES LOOK AT JOURNAL PUBLICATIONS THAT ARE SUBMITTED; AND AS THAT PROCESS TAKES PLACE WITH THE JOURNAL, TYPICALLY OFFICES WILL PROVIDE THE JOURNAL ARTICLE TO PATENT COUNSEL THAT LOOK FOR PATENTABILITY.

NOW, IT'S INTERESTING THAT UNIVERSITIES THEMSELVES DO NOT REALLY LOOK AT FREEDOM TO OPERATE.

THIS IS SOMETHING THAT IS IMPORTANT, I THINK, BUT NONETHELESS DOES NOT OCCUR AT THE UNIVERSITY SETTING, BUT RATHER THE DISCLOSURES LOOK TO SEE IF SOMETHING IS PATENTABLE. NOW, IN THE STEM CELL WORLD, ONE THAT I WAS INVOLVED IN FOR THREE OR FOUR YEARS, THE AMOUNT OF PATENTABLE SUBJECT MATTER IN THE UNIVERSITY SETTING IS ENORMOUS. I USED TO LIKEN IT TO WHEN I WAS IN INDUSTRY LOOKING AT -- IT WAS LIKE MUSHROOMS BECAUSE EVERY DAY YOU'D WAKE UP AND THERE'D BE THREE OR FOUR NEW PATENTS OUT THERE. AND COMPANIES TAKE VERY SERIOUSLY MONITORING INTELLECTUAL PROPERTY BECAUSE YOU HAVE VENTURE CAPITAL FINANCING. AND YOU'RE TRYING TO GET TO MARKET, TRYING TO TAKE YOUR RESPONSIBILITY OF FREEDOM TO OPERATE ALONG THIS LONG, LONG, LONG ROUTE IN THE STEM CELL FIELD IS A BIT -- IT'S A REAL ISSUE. AND I THINK IT'S RELEVANT HERE FOR CIRM BECAUSE REALLY ONE OF THE THINGS THAT WE'RE LOOKING AT IS A HOW TO ADD RESOURCES, ADDITIONAL CAPITAL, IN A WAY THAT OBVIOUSLY HAS THE POTENTIAL TO CREATE EVEN MORE INTELLECTUAL PROPERTY.

BUT NONETHELESS, BACK TO THE UNIVERSITY SETTING, ONCE THE PATENT APPLICATIONS ARE FILED, THEN UNIVERSITIES WILL INITIATE MARKETING ACTIVITIES, THEY'LL FILE A PATENT APPLICATION, AND THERE'S ACTUALLY A LONG PERIOD OF TIME BEFORE THAT PATENT APPLICATION IS PUBLIC, BUT YOU HAVE PROTECTION. AND SO YOU CAN GO OUT TO COMPANIES AND BEGIN

TO MARKET THIS.

NOW, THERE'S ACTUALLY A COUPLE OF SCHOOLS OF THOUGHT THAT HAVE DEVELOPED IN UNIVERSITIES, AND IT RELATES TO HOW THEY MARKET TECHNOLOGIES. I THINK IT DOES HAVE RELEVANCE FOR CIRM AS WELL.

LITA NELSON AND KATHY KU AND OTHERS OF MIT AND STANFORD, I THINK, HAVE DONE -- THEY'RE PIONEERS IN WHAT LITA COULD CALL TECHNOLOGY PUSH. SO YOU GET THE TECHNOLOGY, YOU FILE A PATENT APPLICATION, YOU GO OUT AND REALLY TRY TO LOOK AT LOGICAL LICENSEES, AND THEN YOU TRY TO -- YOU MARKET IT TO THEM AND YOU TRY TO MAKE IT EASY FOR THOSE COMPANIES TO INTEGRATE THE TECHNOLOGY AND ACTUALLY WORK ON IT.

UNFORTUNATELY, IN MY VIEW, THERE ARE MANY, MANY UNIVERSITIES THAT HAVE TAKEN A TECHNOLOGY PULL APPROACH, SO THEY'LL FILE INTELLECTUAL PROPERTY, AND THEN THEY'LL ACTUALLY MAKE IT QUITE DIFFICULT FOR COMPANIES TO CREATE BUSINESS PLANS OF HOW THEY WOULD ACTUALLY EXPLOIT THE TECHNOLOGY WHEN IN MOST CASES THE UNIVERSITY SETTING, THAT THE INVENTIONS ARE VERY, VERY EARLY STAGE. AND SO CERTAINLY ONE OF THE THINGS THAT I BELIEVE -- I THINK IT'S A LITTLE PARADOXICAL, BUT THE TECHNOLOGY PUSH, THEY DO BETTER ECONOMICALLY. IT'S A LITTLE COUNTERINTUITIVE, BUT THE LEADING PROGRAMS REALLY TRY TO FIND GOOD, SOLID COMPANIES AND THEN INCENTIVIZE THEM TO TAKE THAT

TECHNOLOGY AND WORK ON IT. THAT ALLOWS THEM TO DO MORE DEALS AND ACTUALLY THEIR RETURNS YEAR IN AND YEAR OUT ARE MUCH MORE ROBUST. AND I THINK THAT IT'S LOGICAL FOR TWO POINTS TO DO THAT.

THE MECHANISM THAT COMPANIES TYPICALLY USE FOR MARKETING TECHNOLOGIES IS AN OPTION. SOMETIMES YOU GO RIGHT TO A LICENSE, BUT MANY TIMES, ESPECIALLY IN SPONSORED RESEARCH, AGAIN, I THINK THIS IS RELEVANT FOR CIRM BECAUSE YOU'RE BASICALLY GOING TO GIVE COMPANIES GRANTS, SAY $500,000, TO DO A PROTOCOL. AND SO WHAT INTELLECTUAL PROPERTY COULD COME OUT OF THAT RESEARCH. WELL, YOU DON'T KNOW WHEN YOU START THE RESEARCH PROJECT ITSELF, SO TYPICALLY YOU HAVE LANGUAGE IN THERE THAT SAYS A COMPANY WOULD HAVE THE RIGHT TO EXCLUSIVELY LICENSE ANY TECHNOLOGY THAT EMANATES FROM THE SPONSORED RESEARCH. SO YOU DON'T KNOW WHAT IT IS. TYPICALLY IT'S AN OPTION, THE TERMS OF WHICH WILL BE NEGOTIATED AT THE TIME THE INVENTION IS MADE.

NOW, THAT OFTEN -- THAT'S A VERY -- ON ONE SENSE IT'S LOGICAL, BUT ON THE OTHER SENSE, SINCE COMPANIES, ESPECIALLY STEM CELL COMPANIES, WORK IN A HIGH DEGREE OF UNCERTAINTY, ALL COMPANIES ARE TRYING TO CREATE AS MUCH CERTAINTY AS POSSIBLE. SO UNIVERSITIES WILL TYPICALLY SAY, WELL, WE'LL BASE IT ON MARKET PRICES, BUT THE MARKET IS VARIABLE. YOU TRY TO CREATE A RELATIONSHIP WITH A

POTENTIAL LICENSEE OR PEOPLE DOING SPONSORED RESEARCH, BUT THEN, AGAIN, THE COMPANY DOESN'T KNOW IF YOU ARE GOING TO BE IN THAT SPOT OR SOMEONE ELSE. THERE'S THE COMPLEXITY OF THE UNIVERSITY.

SO I THINK THAT THE OPTION APPROACH HAS NOT WORKED AS WELL AS IT OTHERWISE MIGHT, BUT YET I MYSELF HAVE NEVER SEEN TOO POSITIVE. A BETTER WAY TO ACTUALLY DO LICENSING IS A LITTLE BIT MORE DIFFICULT. I THINK I WOULD ECHO WHAT OTHERS HAVE SAID IN TERMS OF WHATEVER MECHANISM IS USED, IT SHOULD CREATE CERTAINTY IN TERMS OF GOING INTO THE RESEARCH AS TO WHAT THE TOTAL EXPOSURE FROM A MONETARY PERSPECTIVE WOULD BE ON A COMPANY AT THE TIME THE PRODUCT IS ACTUALLY MADE.

SO MY OWN COMPANY ITSELF WITH FOUNDATIONS, AND PARENTHETICALLY I THINK THE FOUNDATION, JDRF IS ONE, THE HIGH Q FOUNDATION, THE CYSTIC FIBROSIS FOUNDATION, MANY, MANY OF THOSE FOUNDATIONS ARE REALLY ADOPTING BUSINESS MODELS AND DEVELOPING VERY SOPHISTICATED APPROACHES TO INTELLECTUAL PROPERTY. AND I THINK THAT THERE WOULD BE A RICH DIALOGUE THAT COULD DEVELOP BY REALLY LOOKING ACROSS. I KNOW JDRF IS PARTICIPATING. THEY'RE ONE OF THE LEADERS, BUT OTHER FOUNDATIONS AS WELL ARE REALLY THINKING THROUGH HOW TO DEAL WITH INTELLECTUAL PROPERTY.

BUT I THINK ONE APPROACH THAT HAS WORKED BOTH WHEN I WAS AT CASE WITH COMPANIES AND THEN COMPANIES BACK

WITH THE UNIVERSITY OF MINNESOTA, AGAIN, IS TO CAP YOUR EXPOSURE. BASE IT ON THE PRODUCT AND SAY, OKAY, SO IF $100,000 WAS INVESTED IN FOUNDATION RESEARCH, THEN IF IT MAKES IT TO THE MARKET, THEN A COMPANY WOULD GLADLY PAY X, I DON'T KNOW WHAT THE X WOULD BE, BUT SOME AMOUNT OF SOME TWO TO FOUR X. BASICALLY IT CREATE THE POSITION, THE REAL POSITION, THAT, DEPENDING ON SUCCESS, IT'S POSSIBLE FOR THE ACTUAL INITIAL MONEY TO BE REPLENISHED MANY TIMES OVER. IT HAS ALWAYS BEEN GREETED WHEN I WAS WITH A COMPANY WORKING WITH A FOUNDATION WITH A, YES, THAT REALLY IS SOMETHING THAT MAKES A LOT OF THE SENSE FOR US AS WELL. SO IT WAS REALLY A WIN-WIN IN THAT SCENARIO.

WE'VE EVEN DONE -- WHEN I WAS WITH A COMPANY, WE'VE DONE A ROYALTY BASE. IT WAS A TOOL PATENT, AND MANY, MANY OF THE PATENTS OR THE INTELLECTUAL PROPERTY COMING FROM CIRM ARE GOING TO BE TOOL-BASED PROCESS METHODOLOGIES AND THINGS LIKE THAT. YOU COULD PUT IN A PROVISION FOR A ROYALTY, BUT HAVE A BUYOUT CLAUSE, ESSENTIALLY CONVERT IT TO A FIXED AMOUNT.

THOSE ARE ALL DIFFERENT WAYS TO ADDRESS THE SAME ISSUE, THAT COMPANIES NEED CERTAINTY AT THE END OF THE DAY. THERE'S SO MUCH UNCERTAINTY ALREADY FOR STEM CELL COMPANIES, AND IT'S ONE OF THE REASONS I THINK YOU DO END UP SEEING THE SMALLER COMPANIES. I THINK THAT WE'RE

REALLY KIND OF THE OFFSHOOT OF MONOCLONAL ANTIBODIES, THEN GENE THERAPY COMPANIES, THEN GENOMICS COMPANIES, AND SO THE INVESTMENT CAPITAL IS JUST DEMANDING TO GET IN AND OUT OF A COMPANY IN THREE TO FIVE YEARS. JUST THE TIMING IS -- IT JUST DOESN'T WORK FOR A STEM CELL COMPANY.

SO LASTLY, I GUESS MY LAST COMMENT IS IN TERMS OF WHAT THE BUCK WILL TRY TO DO. I REALLY BELIEVE, HAVING LIVED THIS, IT WAS VERY INTERESTING FOR ME TO SEE OUR SCIENTISTS WHEN I WAS WITH A COMPANY INTERACTING WITH THE UNIVERSITY OF MINNESOTA ON A TANTALIZING ADULT STEM CELL THAT HAD THE POTENTIAL OF DIFFERENTIATING INTO TISSUES FROM THE THREE LINEAGES. AND SO THE ACADEMIC RESEARCHERS WERE VERY, VERY FOCUSED ON THE BIOLOGY OF THESE CELLS. OKAY. AND OUR SCIENTISTS, THEY WERE PEERS, BUT THEY WERE ABSOLUTELY FROM THE VERY BEGINNING INTERESTED IN HOW YOU ACTUALLY COULD MAKE A PRODUCT. HOW THE PROCESS OF, OKAY, IF YOU GREW THEM ONCE, HOW DO YOU GROW THEM A HUNDRED TIMES WHERE YOU HAVE THE SAME OUTPUT? HOW DO YOU KEEP THEM FROM DIFFERENTIATING? WHAT MARKERS COULD BE ASSOCIATED WITH THE ISOLATION?

THOSE TWO THINGS ARE THE EXACT KIND OF ACTIVITY THAT HAS TO HAPPEN VERY, VERY CLOSELY CONNECTED. IN FACT, I WOULD ARGUE IT IS OPTIMAL IF IT'S HAPPENING IN THE SAME PHYSICAL SPACE. SO I THINK THAT IF CIRM COULD REALLY TAKE ADVANTAGE OF SOMEHOW INCENTIVIZING THE

SCIENTISTS WITH DIFFERENT BACKGROUNDS TO COME TOGETHER AND PHYSICALLY WORK TOGETHER, THAT WOULD HELP CONDENSE THE CYCLE TIME FOR MAKING PRODUCTS.

AND THEN THERE'S ONE OTHER COMMENT TO MAKE ABOUT PRODUCTS THEMSELVES IN TERMS OF THE COST OF PRODUCTS. AND SO THE DANGER OF BASICALLY NOT HAVING COMPANIES THAT COME IN AND SAY, OKAY, HERE'S A STEM CELL POPULATION. HOW DO WE MAKE A DOSE? HOW DO WE PUT IT IN A VIAL AND THINK ABOUT DISTRIBUTION AND REALLY MAKE A PRODUCT OUT OF IT? I THINK THAT ONE OF THE THINGS THAT, IN TERMS OF PURE COST, A STEM CELL AS A PRODUCT, SOMETHING IN A VIAL THAT YOU COULD DELIVER, FOR EXAMPLE, IN CONJUNCTION WITH ACUTE MYOCARDIAL INFARCTION, AS OPPOSED TO A PROCESS. RIGHT NOW THERE ARE MANY INVESTIGATORS IN EUROPE IN PARTICULAR THAT WILL DO A BONE MARROW HARVEST IN CONJUNCTION WITH EITHER CONGESTIVE HEART FAILURE OR EVEN A HEART ATTACK, AND THEY'LL FILTER THE CELLS AND BASICALLY DO IT RIGHT AT THE BEDSIDE.

THE TOTAL COST, IF YOU WERE TO TAKE A PROCESS AND THEN TRY TO APPLY IT TO A HUNDRED THOUSAND PATIENTS, AS OPPOSED TO A PRODUCT TO A HUNDRED THOUSAND PATIENTS, IS SO DIFFERENT AND THE PRODUCT WOULD BE SO MUCH BETTER AS A UNIFORM PRODUCT IN A VIAL. IF YOU COULD CALCULATE THE COST DIFFERENTIAL THERE AND BASICALLY SAY THAT WOULD MANY, MANY MORE TIMES MAKE UP MANY TIMES FOLD MORE THAN

TRYING TO COME UP WITH KIND OF A PRICING MODEL, KIND OF ON THE BACK END OF THE MARKET. SO I THINK IT'S REALLY IMPORTANT TO SUPPORT THROUGH CIRM DEVELOPING PRODUCTS AS OPPOSED TO PROCESSES THAT ARE DELIVERED BY CLINICIANS OUT IN THE THERAPEUTIC OR CLINICAL REALM. YOU ARE GOING TO TREAT MORE PATIENTS AND IT WILL BE FAR LESS EXPENSIVE.

SO THOSE ARE MY COMMENTS, AND WOULD LIKE TO THANK EVERYONE FOR INVITING ME AND WOULD BE HAPPY TO TAKE QUESTIONS.

CHAIRMAN PENHOET: THANK YOU VERY MUCH.

(APPLAUSE.)

CHAIRMAN PENHOET: DO WE HAVE ANY QUESTIONS AMONG THE PANEL HERE? ON THE PHONE FROM ELK GROVE? CHICO? BURNHAM?

DR. REED: NO, NONE HERE.

CHAIRMAN PENHOET: IRVINE? LOS ANGELES?

DR. FONTANA: NO, THANK YOU.

CHAIRMAN PENHOET: OKAY. EXCELLENT WRAP-UP. I THINK YOU COVERED -- I'M SORRY. I ALWAYS KEEP FORGETTING THE PUBLIC.

THIS ISSUE -- MAYBE ONE QUESTION. THIS ISSUE OF JUST, IN GENERAL, THERE'S A LOT OF CONCERN ABOUT THE ULTIMATE COST OF STEM CELL THERAPIES AND THIS ISSUE OF PROCESS VERSUS PRODUCT. YOU INDICATED THAT, BASED ON YOUR OWN EXPERIENCE AND SOME CLEARLY ANALYSIS, WHAT MIGHT

THE DIFFERENCE IN COST, EVENTUAL COST TO A PATIENT BE BETWEEN SORT OF A TAILORED THERAPY FOR A SINGLE PATIENT VERSUS A PRODUCT?

DR. KOVACH: YOU KNOW WHAT, I WOULD BE HAPPY TO MODEL IT. I'VE NOT ACTUALLY DONE IT. BUT IT WAS AN ANECDOTAL OBSERVATION AGAIN. OUR INVESTIGATOR WAS WORKING IN CLOSE CONCERT WITH CLINICIANS, IN FACT, WHO STARTED TO DO, IT'S AN AUTOLOGOUS APPROACH IN TERMS OF PROVIDING ADULT STEM CELLS IN CONJUNCTION WITH MYOCARDIAL. THE THOUGHT JUST HIT ME VERY HARD IN TERMS OF HOW DO YOU TAKE THAT OUT TO A HUNDRED THOUSAND PATIENTS AND WHAT WOULD THE COST BE? THE DATA SCATTER AND THE CLINICAL CROWD, THERE'S MANY DIFFERENT ELEMENTS. IT THINK IT WOULD BE A VERY INTERESTING ANALYSIS, BUT IT WAS ONE THAT, SINCE I CAME TO THE BUCK INSTITUTE, I DIDN'T DO ACTUALLY.

CHAIRMAN PENHOET: THANKS VERY MUCH. WELL, GREAT. WE HAVE HALF HOUR LEFT OF THIS MEETING. WE HAVE SCHEDULED ANOTHER MEETING ON APRIL 27TH IN SAN DIEGO WHERE THERE IS ALSO A HIGH CONCENTRATION OF HEALTHCARE COMPANIES OF ONE SORT OR ANOTHER. I THINK WE'VE HEARD A LOT OF DIFFERENT THEMES THIS MORNING.

I THINK ONE THAT WE'LL HAVE TO CONFRONT EARLY ON AND MAYBE EVEN BRING IT UP AT THE NEXT ICOC MEETING ON APRIL 6TH IS THIS ISSUE OF PRIORITIES. WHAT ARE WE

REALLY ATTEMPTING TO DO? ARE WE TRYING TO DRIVE THERAPIES AS RAPIDLY AS POSSIBLE? ARE WE TRYING TO STIMULATE BUSINESS IN CALIFORNIA? DO WE WANT TO GROW SMALL COMPANIES? THERE ARE A WHOLE SET OF POTENTIALLY CONFLICTING AIMS. I THINK AS WE MOVE FORWARD WITH THIS, IT WILL BE VERY IMPORTANT FOR US TO SORT THROUGH THAT AND DECIDE FOR OURSELVES WHICH IS THE MOST IMPORTANT OF THESE OBJECTIVES AND WHICH ARE SECONDARY TO THE MOST IMPORTANT OBJECTIVES.

DR. PIZZO: THAT ONE ACTUALLY GETS RIGHT DOWN TO STRATEGIC PLANNING FOR THE WHOLE OF CIRM. THAT SHOULD BE THE DRIVER, AND THE DERIVATIVE IS WHAT THE APPLICATIONS ARE.

CHAIRMAN PENHOET: THAT'S A VERY GOOD POINT. I ALSO PERSONALLY HAVE SOME EXPERIENCE WITH GOVERNMENT FUNDING IN EUROPE, AND I SAW TIME AFTER TIME COMPANIES ACTUALLY GETTING GOVERNMENT FUNDING FOR THE PROJECTS THAT THEY THEMSELVES WOULDN'T PAY FOR. THEY WERE ALWAYS THE MARGINAL PROJECTS. SO THEY FIGURED, WELL, IF WE CAN GET GOVERNMENT MONEY TO PAY FOR IT, WE'LL DO IT. OTHERWISE WE DON'T DO IT.

DR. PIZZO: WHICH IS ONE OF THE POINTS MADE EARLIER.

CHAIRMAN PENHOET: IT THINK THERE'S A VERY IMPORTANT POINT THAT ALSO CAME OUT OF HERE, NOT TO FUND

THE MARGINAL PROGRAMS FOR US. IT'S HARD ENOUGH WITHOUT --

DR. PIZZO: IT'S A BALANCE ISSUE BECAUSE IF YOU PUT TOO MUCH EFFORT INTO APPLICATION BEFORE YOU'VE GOT THE FUNDAMENTAL KNOWLEDGE, YOU CAN WASTE EVERYTHING.

CHAIRMAN PENHOET: ABSOLUTELY. SO WE DO HAVE AN UPCOMING MEETING. ONE QUESTION IS DO YOU HAVE GUYS AND GALS HAVE SUGGESTIONS ABOUT WHO ELSE WE SHOULD HEAR FROM IN THE NEXT MEETING? ARE THERE GAPS IN WHAT WE HEARD TODAY? WE DO EXPECT TO HEAR FROM THE CYSTIC FIBROSIS FOUNDATION, WHICH HAS BEEN A LEADER AMONG FUNDERS IN FUNDING BOTH THE PRIVATE SECTOR AND THE PUBLIC SECTOR TO DO RESEARCH.

MR. GOLDBERG: I THINK PERHAPS, EVEN THOUGH GENENTECH IS A RELATIVELY LARGE COMPANY NOW, THEY'RE STILL NOT A BIG PHARMA, MAYBE IT WOULD BE HELPFUL, PARTICULARLY IF WE'RE GOING TO BE IN SAN DIEGO, TO HAVE ONE OF THE BIG PHARMAS THAT'S GOT A LARGE RESEARCH PRESENCE IN THE LA JOLLA AREA.

DR. REED: JOHN REED HERE. I KNOW THE SITE HERE IN LA JOLLA FOR PHIZER, KATHERINE MACKEY, IF YOU'D LIKE ME TO EXPLORE THAT.

DR. MAXON: THERE ARE SEVERAL OPTIONS. WE CAN LOOK INTO A BUNCH OF THEM. I'LL CONTACT YOU, JOHN. THANKS.

CHAIRMAN PENHOET: SHOULD WE HEAR FROM SOME STEM CELL COMPANIES? WE'VE HAD A LITTLE BIT OF A BIAS AGAINST THAT GIVEN THE FACT THAT THEY'LL BE DIRECT RECIPIENTS. SO WE TRIED TO HEAR FROM PEOPLE WHO ARE IN THE INDUSTRY, BUT NOT IN STEM CELL COMPANIES. ON THE OTHER HAND, THEY ARE THE ORGANIZATIONS WE'LL BE GOOD DEALING WITH. AN ARGUMENT CAN BE MADE WE SHOULD HEAR FROM SOME OF THEM. WHAT ARE YOUR THOUGHTS ABOUT THAT?

MR. SHEEHY: I THINK THAT WOULD BE GOOD.

DR. FONTANA: I'D LOVE TO HEAR FROM THEM.

CHAIRMAN PENHOET: OKAY.

DR. REED: I THINK THAT WOULD BE WISE.

DR. PRIETO: I THINK IT WOULD BE WORTH HEARING FROM THEM. I'D ALSO BE INTERESTED IN HEARING FROM SOME OF THE OTHER FUNDING AGENCIES, THE NONPROFITS LIKE THE JDRF PRESENTATION WE HAD TODAY, AND ALSO AM CURIOUS WHAT OTHER STATES ARE DOING THAT ARE SORT OF STARTING SMALLER PARALLEL EFFORTS LIKE OURS. I REALLY DON'T KNOW WHAT'S HAPPENING IN NEW YORK, NEW JERSEY, MASSACHUSETTS, ILLINOIS, ETC. AND, FRANKLY, I'D BE A LITTLE CURIOUS HOW THEY'RE LOOKING AT HANDLING THIS.

CHAIRMAN PENHOET: I THINK MARY WOULD PROBABLY BE WILLING TO DO A SURVEY AND PRESENT IT TO US AT THE NEXT MEETING.

DR. FONTANA: ON THAT NOTE, I'D BE INTERESTED IN

HEARING ABOUT SOME OF THE SUCCESSFUL COMPANIES IN SINGAPORE AND HOW THEY INCORPORATED SOME OF THESE ISSUES INTO THEIR POLICIES.

DR. PIZZO: I THINK, ED, IT WOULD ALSO BE POTENTIALLY WORTHWHILE TO HAVE A REPRESENTATIVE FROM CALIFORNIA HEALTHCARE INSTITUTE COME VIS-A-VIS THE DISCUSSION THAT WE HAD EARLIER WITH GENENTECH. TRYING TO GET A BROADER SAMPLING OF HOW THE BROADER BIOTECH COMMUNITY FEELS ABOUT THESE ISSUES COULD BE INFORMATIVE.

CHAIRMAN PENHOET: CHI IS LOCATED IN SAN DIEGO.

DR. PIZZO: SO THAT WOULD BE TIMELY.

CHAIRMAN PENHOET: WE COULD ASK THEM ACTUALLY BETWEEN NOW AND THEN TO POLL THE MEMBERS.

DR. PIZZO: EXACTLY. I ACTUALLY MENTIONED THAT TO STEVE SO THAT WE'D HAVE AN OPPORTUNITY TO DO THAT.

MR. SHEEHY: WOULD WE WANT TO HEAR FROM A VENTURE CAPITALIST? BECAUSE THAT WAS DISCUSSED IN THE CONTEXT OF THE SBIR.

DR. PIZZO: I THINK THAT WOULD BE A GOOD THING ACTUALLY TO DO. MIKE, WHAT DO YOU THINK?

MR. GOLDBERG: I THINK THAT WOULD BE FINE.

MR. SHEEHY: MAYBE BECAUSE IT SEEMS LIKE PART OF THIS RELATIONSHIP WITH FOR-PROFITS IS THE LEVERAGING ISSUE, THAT THE REAL KEY INGREDIENT HERE IS HOW DO YOU MAKE AN INVESTMENT THAT SOMEONE ELSE -- HOW DO YOU MAKE

AN INITIAL INVESTMENT THAT SOMEONE WILL TAKE AND TURN INTO SOMETHING BLOCKBUSTER, AND TRYING TO UNDERSTAND THAT PROCESS.

DR. PIZZO: I THINK IT'S A GOOD IDEA. A PERSON WHO IS INVOLVED IN THIS WOULD BE BROOK BEYER. HE CERTAINLY IS A PRETTY DISTINGUISHED VENTURE CAPITALIST. HE'S WON AN AWARD RECENTLY FROM THE COMMONWEALTH FUND FOR HIS WORK ON STEM CELL OR SUPPORT OF STEM CELLS. SO HE HAS THE KNOWLEDGE AND THE COMMITMENT.

DR. MAXON: I ACTUALLY HAVE A BUNCH OF VC'S LINED UP, AND I HOPE TO TOUCH BASE WITH MICHAEL.

DR. PIZZO: GREAT. THAT WOULD BE A WISE PLAN.

CHAIRMAN PENHOET: AND THEN WE HAD A REQUEST FOR SOME SURVEY DATA BASED ON WHAT OTHER STATES ARE STARTING TO DO. WE DID HAVE FEEDBACK THAT NEW JERSEY HAS JUST PUNTED BASICALLY AND SAID, OKAY, WE'RE GOING TO TAKE A 1-PERCENT ROYALTY ON EVERYTHING AND LET IT GO AT THAT. I DON'T KNOW WHAT THE REST OF YOUR VIEWS HERE. I THINK WE'VE HEARD A LOT OF COMMENTARY TODAY THAT WE NEED A RICHER SET OF CRITERIA AND A MORE FLEXIBLE PROGRAM.

DR. PIZZO: IT'S A SEPARATE ISSUE.

CHAIRMAN PENHOET: WE COULD DEFAULT TO THAT IF WE CAN'T FIGURE OUT WHAT THAT SHOULD BE.

DR. PIZZO: A SEPARATE ISSUE, ED, BUT RELEVANT IS THE WISCONSIN STORY AND HOW THAT'S PLAYING OUT. AND,

YOU KNOW, THOSE ARE AREAS THAT WE DON'T WANT TO GO DOWN. WE DON'T WANT TO GET INTO THE RESTRICTIVE PATENTING THAT THEY'RE DOING. I WONDER WHETHER WE SHOULD HEAR SOMETHING ABOUT THAT AS ONLY A PITFALL TO AVOID.

CHAIRMAN PENHOET: WE COULD INVITE WARF TO COME AND MAKE A PRESENTATION TO US. IT DOESN'T REALLY INFORM OUR POLICY DIRECTLY, I DON'T THINK. EVERYONE DOING THIS RESEARCH HAS TO DEAL WITH WARF, BUT, YOU KNOW, WE DON'T HAVE A DIRECT TIE TO THE POLICY WE HAVE AS A FUNDER IN THAT CASE.

DR. PIZZO: THAT'S TRUE.

CHAIRMAN PENHOET: BUT THERE'S NO DOUBT THAT IT'S AN ISSUE. WE HEARD AT LEAST ONCE, MAYBE TWICE TODAY.

MR. REED: MICHAEL GOLDBERG MADE A SPEECH RECENTLY WHERE HE TALKED ABOUT THE BENEFITS THAT HAVE ALREADY COME TO CALIFORNIA FROM DONATIONS TO THE STATE. THERE'S ALSO A LOT OF SIDE ISSUES THAT ARE COMING, EMPLOYMENT. I WONDER IF THERE'S A WAY WE COULD HAVE SOMEONE SPEAK ABOUT THE POTENTIAL FOR JOBS AND INCREASED TAXES AND THE SIDE BENEFITS.

ONE OF THE THINGS THAT WE DID WITH THE ROMAN REED ACT WAS THAT ONE OF OUR SCIENTISTS JUST CAME UP WITH A NEW PETRIE DISH DESIGNED FOR STEM CELL USE. AND I THINK THERE'S A LOT OF SIDE ISSUES. I'M NOT SURE EXACTLY

HOW TO PHRASE THIS, BUT I THINK THERE'S A LOT OF SIDE BENEFITS THAT ARE NOT BEING TALKED ABOUT THAT COULD BE TALKED ABOUT, EMPLOYMENT, OTHER ISSUES.

CHAIRMAN PENHOET: I THINK THOSE MAY COME INTO FOCUS AS WE TRY TO DECIDE ON THE PRIORITIES. IF IT'S -- I THINK PROBABLY WHAT WE HAVE LEARNED IN THE BIOTECH INDUSTRY IS SUCCESS BREEDS SUCCESS. AND THE FUNDAMENTALS ACTUALLY LEADS, AS A CONSEQUENCE OF THAT, A VIGOROUS ECONOMIC BENEFIT.

MR. REED: THE PRESS IS MOSTLY SEEING BENEFITS IN TERMS OF HOW MUCH IMMEDIATE CASH BACK RETURN. THAT'S HOW IT'S BEING PORTRAYED. I DON'T THINK THAT'S THE PROPER FRAME. I THINK THAT WE NEED TO BE THINKING ABOUT THE EMPLOYMENT, THE NUMBER OF JOBS. I THINK SOMEONE THAT CAN TALK ABOUT THE NUMBER OF JOBS THAT THIS CAN CREATE. I NEVER HEAR PEOPLE SAYING THAT POTENTIALLY BIOTECH IS AS BIG AS AEROSPACE. I KNOW IT'S TRUE, BUT I DON'T HEAR IT. I THINK FOR THE GENERAL POPULATION, WE NEED TO KNOW ABOUT THE OTHER BENEFITS THIS WILL BRING.

MR. GOLDBERG: MAY I SUGGEST THAT A CHI SPEAKER CAN ADDRESS THAT.

CHAIRMAN PENHOET: YEAH. ALTHOUGH I THINK WE'RE GOING TO HAVE THIRD MEETING IN ADDITION TO THE FIRST TWO IN SACRAMENTO, AND THAT MIGHT BE A PLACE TO HAVE SUCH AN OVERVIEW. WE DID DISTRIBUTE, HOWEVER, THE REPORT OF CHI,

ETC., AT THE FIRST IP MEETING IN SACRAMENTO ON THE NONPROFIT STUFF. SO WE DID HAVE SOME OF THAT INCLUDED IN THAT MEETING, THE OVERALL ECONOMIC IMPACT ON THE STATE.

ANY OTHER SUGGESTIONS?

DR. PIZZO: JUST TO THANK YOU AND MARY FOR THIS PROGRAM, WHICH WAS EXCELLENT.

CHAIRMAN PENHOET: ESPECIALLY MARY.

(APPLAUSE.)

CHAIRMAN PENHOET: WE'LL GO OFF THE RECORD.

(THE MEETING WAS THEN CONCLUDED AT 11:41 A.M.)

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 PROCEEDINGS BEFORE THE IP TASK FORCE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE TO THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD AT THE LOCATION INDICATED BELOW

THE GLADSTONE INSTITUTE
1650 OWENS STREET
ROOMS C & D
SAN FRANCISCO, CALIFORNIA
ON
WEDNESDAY, MARCH 29, 2006

WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

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