

Minutes
Health Research Advisory Committee
November 2, 2010
Pennsylvania Health Care Cost Containment Council
225 Market Street, Suite 400
Harrisburg, Pennsylvania

Committee Members Present:

Dwight Davis, MD, Professor, Pennsylvania State University College of Medicine and Director of Cardiac Rehabilitation, Hershey Medical Center
Donna Gentile O'Donnell, PhD, President and CEO, TargetBioToics (morning only)
Michael Huff, RN, Acting Secretary of Health and Chair of the Committee, Commonwealth of Pennsylvania
Arthur Levine, MD, Senior Vice Chancellor for Health Sciences and Dean of the School of Medicine, University of Pittsburgh (via teleconference)
Lewis Kuller, MD, DrPH, Professor of Epidemiology and University Professor of Public Health, Graduate School of Public Health, University of Pittsburgh (via teleconference)
Michael Parmacek, MD, Herbert C. Rorer Professor of Medical Sciences and Director of the Penn Cardiovascular Institute, University of Pennsylvania School of Medicine
Kim Smith-Whitley, MD, Assistant Professor, Department of Hematology, The Children's Hospital of Philadelphia
Lisa Staiano-Coico, PhD, President, The City College of New York (via teleconference, afternoon only)

Department of Health Staff:

Cathy Becker, MPH, Health Research Program Manager, Bureau of Health Statistics and Research
Christine Dutton, Esq, Chief Legal Counsel
Dwayne Heckert, Legislative Specialist
Diane Kirsch, RHIA, CTR, Public Health Program Administrator, Health Research Program, Bureau of Health Statistics and Research
John Koch, Program Analyst, Health Research Program, Bureau of Health Statistics and Research
Patricia W. Potrzebowski, PhD, Director, Bureau of Health Statistics and Research
Robert Torres, JD, Deputy Secretary for Administration
Mackenzie Wroble, Intern

Others in Attendance:

John Anthony, Project Associate, Pennsylvania State University
Katrina Armstrong, MD, MS, Professor of Medicine, Director, Division of General Internal Medicine, University of Pennsylvania
James E. Barrett, PhD, Professor and Chair, Drexel University
Kara D. Beem, Esq, Senior Associate, Greenlee Partners, LLC
Mel Billingsley, PhD, President and CEO, Life Science Greenhouse of Central Pennsylvania

Morris J. Birnbaum, MD, PhD, Professor of Medicine, University of Pennsylvania
Sheilah Borne, Director, Government Relations, Pennsylvania State University
Paul Cribbons, Esq, Assistant Vice President, Office of Government and Community Affairs,
University of Pennsylvania
Jeanne Cunicelli, MBA, Investment Partner, Bay City Capital (via teleconference)
Prudence W. Dalrymple, PhD, MS, Director, Institute for Healthcare Informatics, Drexel
University
John Giannelli, Government Relations, Obermayer Rebmann Maxwell & Hippel, LLP
Jean Givey
Angela E. Gohn, Associate, Greenlee Partners, LLC
Marc Malandro, PhD, CLP, Associate Vice Chancellor for Technology Management and
Commercialization and Director, Office of Technology Management, University of
Pittsburgh
Margaret C. McDonald, PhD, MFA, Associate Vice Chancellor for Academic Affairs, University
of Pittsburgh
Robert McGrath, PhD, Executive Director, Entrepreneurship & Technology Commercialization
and Associate Vice Provost, Drexel University
Thomas Penn, JD, MBA, General Partner, Meridian Venture Partners
Linda M. Siminerio, PhD, Executive Director, University of Pittsburgh Diabetes Institute
Neal Simon, PhD, Chief Executive Officer, Azevan Pharmaceuticals
Joy Soleiman, MPA, Clinical Administrator, Jefferson Kimmel Cancer Center, Thomas Jefferson
University
David Wilson, Assistant Vice President, Governmental and Community Relations, Drexel
University

Call to Order

The Chair, Acting Secretary of Health Michael Huff, called the meeting to order at 9:05 a.m. on Tuesday, November 2, 2010 in the 4th floor conference room of the Pennsylvania Health Care Cost Containment Council in Harrisburg, Pennsylvania. A quorum of members was present in person or via telephone. Mr. Huff welcomed Committee members and others to the meeting. He announced that the purpose of the meeting was to hear recommendations from invited speakers regarding potential health research priorities and to discuss and determine the areas of research to be considered for next year's priorities.

Minutes of the February 8, 2010 Meeting

Dr. Gentile O'Donnell objected to the wording of the minutes, in particular the motion on page 11. Mr. Huff tabled the approval of the minutes until the next meeting which would give staff an opportunity to recheck the recording of the meeting.

Program Update

Mr. Huff stated that handouts summarizing program accomplishments during the past year and measures of success were emailed to the committee prior to the meeting.

Commercialization Workshop

Robert Torres, Deputy Secretary for Administration, stated that during its last meeting the committee recommended that half of the nonformula funds for 2011-12 be invested in research on the discovery or development of drugs, devices, diagnostics, vaccines and health informatics with commercialization potential. Speakers from university technology transfer offices and speakers who invest in companies or work with companies on commercialization were invited to present their view of research opportunities. Dr. Parmacek requested that speakers address how this priority addresses all of the requirements for research in Act 77. Mr. Torres clarified that the speakers were asked to focus on how to optimize the funds for this type of research and they may not be familiar with the Act. This question could be addressed after the presentations during the committee's discussion of the commercialization issues.

Presentations and Questions and Answers

- Ms. Jeanne Cunicelli from Bay City Capital indicated that an investment of \$500,000 - \$1 million could be used productively to develop proof of concept by supporting animal studies and Phase I and II studies, but not Phase III studies, which are cost prohibitive. This level of funding could also be used for transferring compounds directly to manufacturers and supporting an Investigational New Drug (IND) process. Dr. Parmacek commented that Act 77 requires that we fund research and not commercialization activities. He also raised questions about funding the development of devices.
- Dr. Robert McGrath from Drexel University stated that the university has spun out about 20 companies based on early stage technologies during the past five years. He explained that a good proposal should have strong proof of concept data, a realistic and detailed research program with defined milestones, evidence of solid intellectual property, a realistic, milestone-based plan for obtaining post grant funding and a technology that addresses a high value market or an unmet need. He recommended funding in stages, quarterly reporting, periodic review panels, and a separate proposal evaluation panel consisting of industry representatives and technology transfer professionals to complement the scientific review. In response to questions, Dr. McGrath indicated that it may take 36 months for a company to take a device in animal or human studies to market and 10 years to take a pharmaceutical to market. Drexel's translational research partners program is less science intensive than an NIH Small Business Innovation Research (SBIR) process. Dr. Parmacek questioned whether or not the proposed research priority would include universities. Dr. Gentile O'Donnell commented that she indicated during the February committee meeting she was very much in favor of including universities. Dr. Davis added that there are well aligned partnerships between companies and universities which allows for significant leveraging. Mr. Huff asked the Department of Health's chief counsel Christine Dutton to clarify the question concerning whether or not funding can be restricted to companies. Ms. Dutton indicated that the motion passed at the February meeting restricted funding to companies, but the Department of Health (Department) cannot limit funding to companies. Dr. Smith-Whitley expressed concern about the review process proposed by Dr. McGrath because of conflict of interest issues. Dr. McGrath responded that he would exclude himself from any discussion of a Drexel University proposal. Dr. Kuller commented that a fair amount of formula funds has been provided to Drexel and other universities and asked how much money Drexel has used for their technology transfer research. Dr. McGrath indicated that no formula funds were

used because funding is provided by the Wallace Coulter Foundation. Dr. Kuller asked why nonformula funds are needed now if formula funds were not needed. Dr. McGrath responded that there is so little money available for investment funding. These funds are the riskiest investments because a technology can fail during studies undertaken to move the technology forward.

- Dr. Marc Malandro from the University of Pittsburgh summarized his university's resources and record of technology commercialization. He stated that he is a proponent of public-private partnerships for commercialization because each group working without the other is not as likely to succeed. In response to Dr. Davis's question about lessons learned during the downturn in the economic environment, Dr. Malandro indicated that there has been a large reduction in funding for early stage research in favor of clinical development and marketing. There is a bigger upswing in investment in medical device development. In response to Dr. Gentile O'Donnell's question, Dr. Malandro indicated that he would be willing to serve on a review committee at no expense to the Commonwealth.
- Mr. Thomas Penn from Meridian Venture Partners indicated that \$500,000 in grant funding would be useful for obtaining capital equipment and pre-clinical development. He provided examples of how three companies might use the funding. He concluded with the recommendation that the grants program be kept simple with minimal strings attached. Dr. Davis asked about the likely relationship between the company and the commonwealth after the grant ends. Mr. Penn envisioned that a grant program with non-dilutive funding would likely not return any investment to the commonwealth, but that issue would need to be resolved.
- Dr. Neal Simon from Azevan Pharmaceuticals provided information on his company and noted that non-dilutive NIH and other federal funds enabled his company to obtain venture capital backing. His company has also benefited from a partnership with Lehigh University. In the current climate new technologies must be "de-risked" through the provision of proof of concept data in order to obtain venture capital funding. He offered two alternative models for awards, (1) \$500,000 per award or (2) awards for preclinical studies at \$300,000 per project and awards for clinical studies at \$700,000 per project. Qualifications for the grant might include a record of current or previous SBIR Phase II support (Phase I alone is insufficient) and/or venture capital funding that exceeds a certain level.

Issues Regarding the Commercialization Priority for 50% of 2011-12 State Fiscal Year (SFY) Nonformula Funds

Mr. Torres stated that the Department emailed to the committee on July 6th an update on activities undertaken by the Department since the February 8th committee meeting to determine how to review and fund research projects with commercialization potential in a cost efficient and effective manner. The email raised issues for the committee's consideration at this meeting. Prior to this meeting the committee was also sent a draft of the commercialization research priority. Mr. Torres summarized the issues as follows:

- The first issue concerned the amount of the grant award. In the July email, it was recommended that the size of the awards be limited to a maximum of \$750,000. If we assume the same level of tobacco settlement funding as this current year, between \$7 and \$8 million would be available to fund projects to address this particular priority. At \$750,000 per project we could fund between 10-12 projects.

- At the last meeting there was discussion on collaboration. It was recommended that the requirement for collaboration be dropped in order to allow private sector firms to compete on their own accord.
- The third issue was proof-of-concept. There was a concern than roughly half of the Small Business Innovation Research grants funded by NIH are not successful. If proof-of-concept is required, it was suggested that this might increase the likelihood of success of our projects.
- The fourth issue had to do with including commercialization information in the application. It was proposed that such information include commercial viability and plans, marketing management, other sources of money for the project and company management.
- The fifth issue was narrowing the focus of the research priority. The motion in February was fairly broad and there was question as to whether or not the priority should be narrowed to a health issue such as cancer and heart disease or other area of interest to the committee.
- There was some discussion about restricting bidding to companies. Staff discussed this issue with the Department's chief counsel and we believe we cannot restrict the bidding according to the Act. To do anything differently would put the Department at risk.

Mr. Torres then called for comments and discussion on the issues.

Discussion

Maximum limit on grant awards – Dr. Gentile O'Donnell commented that she agreed with the proposed \$750,000 limitation on the amount of the award, but she also liked the proposed alternative of providing smaller awards to early proof of concept studies and larger awards to clinical studies.

In response to Dr. Davis's question about the expected return on these funds, Dr. Parmacek commented that to be consistent with the spirit of the legislation regardless of how the grants are structured, the desired return should be the impact on public health and the health of Pennsylvanians rather than a financial return on investment.

Mr. Huff suggested that language concerning impact on public health should be included in the wording of the priority.

Dr. Gentile O'Donnell proposed that peer review be done using a different approach. Mr. Huff asked Ms. Dutton to provide an overview of what is allowed and not allowed by the legislation.

Ms. Dutton indicated there are two issues related to peer review. There is the Tobacco Settlement Act with its requirements for the composition of the peer review panels and how the review is to be done. There are also the state Adverse Interest Act and the state Ethics Act. This committee is an advisory committee and cannot become involved in the details of how grant proposals are reviewed. She cautioned that if a committee member does this, the member is taking the risk that any entity that the committee member is associated with can be disqualified from bidding. Furthermore, the Tobacco Settlement Act itself states that an employee of the

applicant whose proposal is under review cannot serve on a peer review panel. One of the reasons why the Department uses out-of-state peer reviewers is that it makes clear that the Department's employees and the members of this committee are not interfering in any way in the objectivity and the ultimate decisions that are made on the grant proposals. If the committee focuses on the priorities, which this committee has been specifically charged to do in conjunction with the Department, the committee will be safe. The composition of the peer review panels, the peer review processes and how the Department awards the grants are not under the committee's purview.

Dr. Gentile O'Donnell expressed concern that Oak Ridge Associated Universities (Oak Ridge) cannot manage the review in a cost effective manner and that there are Pennsylvania companies which are focused on technology transfer and that could do the review instead. Mr. Torres acknowledged that there will be significant costs because the priority as it now stands is very broad. The Department held several discussions with Oak Ridge regarding how to make the review more efficient and the subject matter expertise and competencies of reviewers that they had access to. He said that the Department was satisfied that Oak Ridge can respond to the Department's requirements regarding how the review process should be managed.

Dr. Gentile O'Donnell recommended that some other alternative methodology that won't cost a lot be developed and she asked that the Department obtain an opinion from the Ethics Committee. Ms. Dutton indicated that the Ethics Committee does not administer the state Adverse Interest Act which is the primary Act of concern here and an opinion of the Ethics Committee would not allow the committee to discuss the process by which grants are selected. The Department also has a contractual agreement, a bid was issued and Oak Ridge was selected as the contractor. Their contract is in effect until 2013.

Dr. Kuller commented that Oak Ridge won a competitive bid and a sudden change to a new review system is concerning. All researchers experience the same problem, that is, when they submit a proposal that doesn't receive funding, they criticize the reviewers. The reality is that the review process works. He added that the Department should decide the fairest way to review these grants. If the Department determines that Oak Ridge can't do the review, then the Department should decide who should do the review. The committee should not decide how proposals are reviewed.

Dr. Parmacek pointed out that if the priority is limited in some way the decision about how to review it might be easier. If the committee is comfortable with funding 10 projects, \$750,000 would probably be a reasonable limit to the grant award.

Dr. Parmacek asked whether the grants would be for a four-year period. Dr. Potrzebowski replied that there was some discussion with the life science greenhouses and one issue was that the funds would be needed more quickly and so the grants might be for a two-year period.

Dr. Smith-Whitley brought up the question of whether or not profitability should be included in the wording of the priority. She indicated that sustainability is important and we should choose technologies that are likely to go forward. There are many examples of research on orphan diseases that do not go forward because of lack of interest or marketability.

Collaboration – Mr. Torres reiterated that Act 77 requires that there be no restrictions on applicants. Bidding for research funding on this priority would be open to individual companies, companies in collaboration with universities, or any eligible applicant. Dr. Davis asked whether the requirement for collaboration would be dropped for other priorities. Dr. Potrzebowski indicated that the recommendation to drop the requirement for collaboration was made for this particular priority only.

Proof of concept – Mr. Huff observed that presenters were defining proof of concept differently ways and asked how it should be defined. Dr. Potrzebowski remarked that we have asked for preliminary data to show proof of effectiveness in the past and suggested that these projects would be equivalent to a SBIR Phase II or later phase award. Dr. Davis asked whether the definition would be included and indicated his support for requiring some evidence of the viability of the technology. Dr. Parmacek commented that a simple way to distinguish would be clinical vs. pre-clinical proof of concept. He added that the SBIR language would be helpful in developing the definition. He suggested that this priority could target therapeutic molecules, biomarkers/diagnostics and devices, but argued against vaccines because they were included in a prior priority. He stated that he would favor going more heavily toward the clinical as opposed to pre-clinical proof of concept.

Dr. Kuller asked what product was anticipated from this priority. He indicated that Phase III and IV studies should have priority over Phase I studies. Dr. Gentile O'Donnell disagreed and commented that the large pharmaceutical companies will pick up funding for Phase III and IV studies whereas the problem regarding lack of funding lies with the early stage development technology where a little bit of funding to bench researchers will move the technology along the pipeline. Dr. Kuller disagreed and stated that there are examples of products such as Pittsburgh compound B and the same product developed at the University of Pennsylvania that offer huge advances in studying the brain. The big problem is the cost of doing the needed studies and large companies are not responding with funding.

Mr. Torres summarized that our ideas regarding proof of concept would be equivalent to any project that is in SBIR Phase II and beyond. Dr. Parmacek added that SBIR Phase II grants are in the same funding range as what we proposed and one of the problems with Phase III and IV studies is their cost.

Inclusion of financial and commercial information – Mr. Torres stated that commercial and financial information and information on sustaining the project after the grant ends should be included in the proposals. The Department incorporated recommendations from the life science greenhouses regarding the needed information. Mr. Torres stated that the Department would provide this information to the committee.

Dr. Parmacek asked if there was a restriction regarding where the corporate headquarters are located, i.e., can companies with corporate headquarters located outside the state receive funding if they have discovery centers in Pennsylvania? Mr. Huff stated that the Department would provide that information to the committee. Dr. Davis commented that there might be questions in

the future if our funding leads to significant profits for companies located outside of Pennsylvania.

Narrowing the focus of the priority – Dr. Parmacek commented that vaccines should probably be excluded because this area of research was funded in the past and suggested that the committee choose one topic from among several suggested: therapeutics, diagnostics, devices, biomarkers or new gene therapies. Dr. Smith-Whitley proposed consideration of health informatics since this is the era of electronic health records. Dr. Levine stated he is concerned how the review will be handled if any one of the three areas is selected. Unless the focus is narrowed to, for example, cancer diagnostics, diabetes therapeutics or cardiovascular devices we would be overwhelmed during any review process. Dr. Gentile O'Donnell indicated that she did not know how a large universe of great ideas in tech transfer can be captured if we narrow the focus before the review process is begun. It will not be in the best interest of early stage research. Dr. Levine remarked that the amount of money available is small, 10 grants at \$750,000 a piece, and it will not cover the whole of technology emergence in the commonwealth and unless we prioritize there is no existing review process that can address every proposal that is submitted, even if it is parsed into diagnostics, therapeutics and devices. Dr. Gentile O'Donnell responded that this was the reason why she proposed an alternative mechanism to do a preview of the universe. Dr. Levine responded that he knows of no review process that would allow discrimination between developing therapeutics or diagnostics for any of 45 different diseases and/or 6,400 orphan diseases. Dr. Gentile O'Donnell recommended a review process consisting of people who do this, such as people from the technology transfer office at the University of Pittsburgh and others. Dr. Levine replied that he is not aware of any review consistent with the Act that would allow an ad hoc review process, e.g., if we received 100 proposals for 100 different areas of medicine it would require setting up expert review panels for each different area. If the priority is narrowed to cancer diagnostics, diabetes therapeutics or cardiovascular devices, it would be easy for Oak Ridge or a similar company to conduct the peer review. Dr. Gentile O'Donnell requested a copy of the Oak Ridge contract.

At this point the meeting was suspended for lunch.

Invited Testimony on Research Priorities for State Fiscal Year 2011-2012

When the meeting was reconvened, Mr. Torres explained that the Department invited speakers, selected by the Committee based on written testimony submitted to the Department, to present ideas on potential research priorities for the other half of the nonformula funds. The names of the presenters, a brief summary of the research priority presented, and responses to questions raised by the Committee are summarized below.

- Dr. Katrina Armstrong from the University of Pennsylvania stated there has been a huge investment in medical research on genetic variants which are associated with diseases. Now there is a need for translational research to determine the clinical utility of these genomic discoveries for decision making with individual patients, providers and policy makers. Such research would determine which tests are needed to improve clinical outcomes and which patients might need or not need the tests. In response to Dr. Parmacek's question as to how would our funding differ from NIH's initiatives, Dr. Armstrong replied that NIH funds

genomics research to discover genetic factors associated with disease, but does not support studies on how to use this information in terms of diagnostic testing. Also, there is a large federal investment in comparative effectiveness research, which compares the impact of drugs or interventions on large study populations without addressing the issue of risk assessment or diagnostic testing. This proposed research would fill the gap between genomics discoveries and comparative effectiveness research. Dr. Levine agreed that there is a gap and need for the proposed research. Dr. Davis commented that there are a lot of genetic discoveries and it is difficult to translate all of these discoveries into clinical practice, but research on clinical utility is potentially huge. Dr. Levine responded that the availability of information on the relationship of genetic factors to breast cancer has changed the way breast cancer is managed in patients. Because the size of the research area is large, the committee should focus on a particular area where there is insufficient translation to clinical practice. Dr. Kuller agreed with the need for this type of research and commented that the differences in mortality due to breast cancer between black and white women was originally thought to be attributable to differences in screening and is now thought to be attributable to the differences in genetic factors. A consideration of these factors would lead to improved screening, treatment and better outcomes for black women. The area should not be broad based, i.e., genomics research, but targeted to very specific areas of cancer for example. Dr. Smith-Whitley mentioned that this type of research requires large study populations which presents a challenge for short funding periods. Dr. Armstrong replied that research can be done with existing data and gave as an example the issue of conducting biopsies on women with BI-RADS 4 mammograms when the risk of cancer might vary from 1% to 70% depending on genetic risk factors. Such a study might only require the collection of DNA.

- Dr. Linda Siminerio from the University of Pittsburgh stated that diabetes has reached epidemic proportions and its complications are extensive and expensive. Despite advances in understanding the disease and therapeutic approaches, patient outcomes are poor and the number of practitioners is shrinking. Research is needed to evaluate telemedicine care alternatives, the effect of age and duration of diabetes on patient adherence to recommended therapies and the development of new treatment approaches. In response to a question of how this priority should be focused so it would be distinguished from the NIH's initiatives, Dr. Siminerio stated that the likelihood of NIH funding is diminishing. Their institution has good research on insulin resistance, genetics, adherence and technological approaches, and researchers need resources to continue their work.
- Dr. Morris Birnbaum from the University of Pennsylvania indicated that obesity, which affects two-thirds of Pennsylvanians, is a predisposing factor for type 2 diabetes. There is a need for a better understanding of why obesity causes insulin resistance in diabetes, what it is about the fat cells that increase the likelihood of diabetes and cardiovascular disease, and how genetic factors affect individual's response to different therapies. Recent large collaborative studies found that improving blood glucose improved the symptoms of diabetes but did not reduce cardiovascular disease and in one study actually increased cardiovascular disease. These findings raise questions about the current approach to the treatment. He added that the NIH doesn't fund pilot studies and, in particular, the transition from pre-clinical (animal) studies to clinical (human) research.
- Dr. Prudence Dalrymple from Drexel University advocated for funding of health informatics research. Examples include GIS mapping to identify factors related to health disparities, studies of devices and feedback systems to promote healthy lifestyles, evaluation of

telehealth applications, evaluation of best practices for data analysis and development of new ways to encrypt data. Dr. Parmacek noted that the implementation of electronic medical records (EMR) at the University of Pennsylvania added 10 minutes to the physician's work and this was work previously done by non-physicians. He recommended that the impact of EMR on the efficiency of care be considered as an area of research if this is selected as a priority.

- Dr. James Barrett from Drexel University stated that cancer pain has a profound impact on patients, their families and the health care system. Cancer pain originates from cancer itself and cancer therapies. In many instances there are no effective pain treatment options. Research is needed to gain a better understanding of the mechanics associated with cancer pain that could lead to the discovery of better treatments. Dr. Davis questioned why the National Cancer Institute (NCI) has not invested significant funding in this area. Dr. Barrett replied that the NCI is focused on cancer itself. The National Institute on Drug Abuse (NIDA) had provided some funding for opioid research, which is the primary standard therapy. Many of the current treatments are over 100 years old.

Draft Commercialization Priority

Mr. Torres stated that the draft commercialization priority was emailed to the committee prior to the meeting and asked for comments.

Dr. Parmacek recommended that the scope be limited, for example, to cancer diagnostics. Dr. Kuller recommended that cancer therapeutics be added to cancer diagnostics because there is a great need and there are small businesses in eastern and western Pennsylvania doing research to identify markers for drug response and high risk. Dr. Davis suggested that the focus be narrowed to one area or the other. Dr. Levine agreed that the scope should be as narrow as possible. He commented that the review process should include peer reviewers who can evaluate scientific merit and reviewers who can evaluate the business/economic value of the proposals. Mr. Huff supported a focus on cancer diagnostics because of the potential for public health prevention. Dr. Parmacek indicated that therapeutics is very broad and includes cell therapy, biologics, gene therapies and vaccines, whereas diagnostics is an exciting area and there is a need for prognostic, diagnostic and therapy driving markers. Dr. Kuller suggested surveying PA BIO to obtain a list of Pennsylvania companies that conduct research in each of these areas before determining the focus of the priority. Dr. Levine commented that there is a real issue with diagnostics because there are a substantial number of companies that are proposing antigens, polymorphisms, mutations or other molecular variants that predict a particular therapy and most are not founded in evidence so we could make a contribution by funding companies that are focusing on evidence-based diagnostics. Dr. Kuller added that Medicare and other payers are now requiring proof of principle that these diagnostics improve prognosis and outcome.

Dr. Potrzebowski indicated that the Department will survey PA BIO and the life science greenhouses to obtain a list of Pennsylvania companies that conduct research on cancer diagnostics that lead to a particular therapeutic strategy, cancer therapeutics and neurosensory diagnostics.

Dr. Potrzebowski summarized the discussion from the morning's session regarding the language that should be included in the priority. i.e., that the research should have a significant impact on the health of Pennsylvanians and the potential to improve the health of Pennsylvania citizens and that the research project should have sustainability. The examples of research "to be included but not limited to" would be revised after the committee recommends the focus of the priority. Dr. Levine commented that the research should also be expected to improve the social (economic) health of Pennsylvania. He suggested not making collaboration with universities a requirement but stating that there will be a particular interest in companies that have a university association. Dr. Staiano-Coico asked how this funding dovetails with NIH's SBIR funding since this appears to be a very similar mechanism. Dr. Potrzebowski stated that based on earlier discussions we are looking for projects that are SBIR Phase II or later.

Tobacco Settlement Act (Act 77) Requirements for Setting Priorities

Mr. Torres pointed out that the committee received a handout outlining the responsibilities of the Department for setting priorities in section 904 of the Tobacco Settlement Act. This handout also listed the guiding principles that were established by the committee in 2003.

Research Priorities for Formula Funds for State Fiscal Year 2011-12

Dr. Parmacek moved that the formula priorities remain the same as in past years. Dr. Davis seconded the motion and all committee members present in person or on the phone voted in favor of the motion. The research priorities for the formula funds for SFY 2011-12 are:

Research priorities shall include the identification of critical research areas, disparities in health status among various Commonwealth populations, expected research outcomes and benefits and disease prevention and treatment methodologies.

The research priorities are clinical, health services, and/or biomedical research as defined in Act 2001-77. The ultimate goal of the research should be to improve health status and access. The Department should encourage, through the application process and accountability requirements, research that:

- emphasizes collaboration
- promotes business and community involvement
- increases infrastructure and research capacity
- increases the number of new investigators, new grants, new discoveries and new products
- leverages new and existing research funds, and
- leads to population-based applications that address disparities in health status among various Commonwealth populations.

An institution that receives \$400,000 or more in formula funds shall also comply with the requirements of Section 908 (c) of Act 2001-77.

Institutions receiving grants under Section 909 of Act 2001-77 shall also comply with the requirements of Section 910 of Act 2001-77.

Research Priorities for the Other Half of the Nonformula Funds for State Fiscal Year 2011-2012

Mr. Torres asked the committee for their ideas on priorities for the remaining 50 percent of the nonformula funds.

Dr. Levine proposed the nexus of genomics and comparative effective research on cancer. In response to Dr. Kuller's question, he confirmed that this would include somatic retentions in cancer cell lines, as well as germline abnormalities. Dr. Levine clarified that he is proposing research to fill the gap between the comparative effectiveness research funded by the Agency on Health Research and Quality (AHRQ) and research funded by the NIH. AHRQ is focused on meta analysis, systematic reviews and clinical trials and the NIH is focused on genomics. Dr. Kuller recommended defining comparative effective research as application of genomics in evaluating responses in clinical trials. Otherwise, we might receive proposals for meta analyses. Dr. Levine suggested the definition contained in the Institute of Medicine (IOM) volume on initial national priorities for comparative effectiveness research. Researchers could propose a prospective clinical trial or research which involves retrieving genomics tissue bank data for patients in clinical trials that have already been completed.

Dr. Parmacek pointed out that we heard from two speakers on diabetes and this is one area that has not been funded. If genomics and translation of genomics is selected as the priority this year, he asked that diabetes be considered next year. Understanding the fat cell's function in diabetes is important because treatment of blood sugar may be the wrong approach. Mr. Huff reinforced the need for diabetes research because diabetes is an important public health need in Pennsylvania. Dr. Kuller commented that there is a tremendous amount of funding from NIH and private companies for new drugs, but that it might be a good idea to invest all of the funds in diabetes next year. On a related note, he mentioned that the amount of bariatric surgery in Pennsylvania is phenomenal especially since there is no research on long term outcomes. Dr. Parmacek suggested a short, 2-hour workshop on diabetes research at the first meeting next year.

Mr. Torres summarized the discussion by saying that he understood that the priority should be on translating genomics with a focus on cancer. Dr. Parmacek nominated Dr. Armstrong to work with Dr. Levine on drafting the white paper to be presented to the Department by the 19th of November.

Next Meeting

The next meeting will be held on December 8th from 9:00 a.m. to 4:00 p.m. The meeting will take place in the Bureau of Health Statistics and Research. The committee will hear progress reports on the 2007 nonformula grants on violence prevention and regenerative medicine. The other purpose will be to determine the other nonformula research priority and finalize the commercialization priority.

Dr. Kuller noted that the University of Pittsburgh's obesity project funded by nonformula funds was the prime paper and focus of a major, very positive editorial in a recent issue of the Journal of the American Medical Association (JAMA).

Adjournment

The meeting adjourned at 2:40 p.m.