

# SMD RESEARCH STRATEGIC PLAN

## *Executive Summary, Feb. 2014; Updated July 2014*

**Research Vision:** To improve the health of the community through research and discovery

**Research Goal:** Create a sustainable research enterprise focused on programs of excellence that feature cutting edge science, have national/international prominence, and dovetail with our clinical areas of excellence.

**Overview:** In 1996, the URM C embarked on an ambitious strategic plan designed to increase the research enterprise by recruiting  $\approx 75$  faculty and erecting a new research building to house this faculty. Based upon the success of this strategic plan, in 2007 the URM C embarked on a second strategic plan that also focused on continued growth of the research enterprise, with metrics also based upon faculty recruitment and NIH funding. New recruitment was to focus on five integrated disease programs (IDPs: Cancer, Cardiovascular Diseases, Immunobiology and Infection, Musculoskeletal Diseases, Neuromedicine) that were designed to create bridges between the basic and clinical sciences and to take advantage of areas of clinical strength.

As a result of these two strategic plans, we increased our research faculty by  $\approx 200$ , built three research buildings (Kornberg MRB, DelMonte Neuromedicine Institute, and Saunders Research Building), purchased the Wyeth Building in Henrietta to house the Aab Cardiovascular Research Institute (CVRI), and renovated considerable space at the medical center. As a result,

we now have one of the largest research enterprises in faculty size and research space among US academic medical centers. Although NIH funding more than doubled, our rank in total NIH funding remained unchanged, because most of our peer institutions experienced similar growth in research. In contrast to our peers, however, we demonstrated an unrivaled

	1996	2007	2013
<b>Total Award Count</b>	658	1,520	1,331
<b>NIH Award Count</b>	253	451	393
<b>Total \$'s Awarded</b>	\$90,857,149	\$244,849,174	\$199,683,043
<b>NIH \$'s Awarded</b>	\$60,457,715	\$161,840,899	\$132,414,203
<b>Basic Science Faculty</b>	111	175	172
<b>Tenure Track Faculty</b>	537	824	816
<b>PI's on Grants</b>	335	612	573
<b>Research Space (ft<sup>2</sup>)</b>	303,512	536,392	643,968

growth in publication citations, such that the UR entered the top 20 in this metric. The table summarizes the changes in our research enterprise since the onset of the 1996 Strategic Plan.

The NIH budget doubled over the period 1998 to 2003, but this has been followed by a prolonged flattening, the length of which (>10 years) has been unprecedented - resulting in a precipitous drop in the success of all grant applications from  $\approx 35\%$  to  $< 15\%$ . With this reduction, the extent to which researchers can support their salaries has fallen substantially and thus the required institutional support has grown from  $\approx 25\%$  to  $\approx 45\%$  of that provided by the NIH and other government agencies. Continued pressure to reduce the federal budget will likely present substantial challenges to biomedical research in the years ahead.

Given the size of our current research enterprise, the effective decrease in extramural funding, and our relatively low endowment, we need to develop a research strategic plan that focuses more on enhancing the programs of our current investigators - rather than on the development of new programs and continued expansion of the faculty. To ensure continuing

vibrancy, this plan will also need to provide for future recruitments in targeted areas of strength - with the goal of strengthening existing programs and creating new opportunities for research collaboration that will benefit many investigators.

Our new strategic plan therefore focuses on approaches that **enhance our national and international reputation and also enhance our competitiveness for funding** by maximizing collaborations among faculty across URMC departments and centers, as well as with faculty in other units of the UR. These collaborations will also take advantage of our position as a large and outstanding clinical enterprise to develop interdisciplinary programs that will most effectively compete for funding for clinical and translational research. The centerpiece of this strategic plan will therefore be the fostering of “programs of excellence” throughout the research enterprise. The research strategic plan has four major goals:

1. To promote **research programs of excellence**. A key objective of these programs is to support our current productive faculty, and to enhance their research competitiveness by promoting scientific communities and encouraging interdisciplinary collaboration.
2. To identify key **core resources** that will be necessary to promote these programs and assure that we can perform state-of-the-art cutting-edge science.
3. To implement a **research infrastructure** best suited to advancing our research priorities.
4. To implement a **sustainable financial model** that will enable us to achieve our strategic goals.

## **1. TO PROMOTE RESEARCH PROGRAMS OF EXCELLENCE.**

Our strategic plan focuses on **enhancing the national/international reputation of the UR and URMC** by supporting and promoting our research programs of excellence (basic, clinical, and translational). The organization of our research enterprise into programs of excellence is designed to: improve efficiencies, promote collaborations among investigators throughout the medical center, the river campus and other institutions (including the private sector); promote interactions among basic scientists, clinical investigators and clinicians. *A key overarching objective of these programs is to support our current productive faculty, and to enhance their research competitiveness by promoting scientific communities and encouraging interdisciplinary collaboration.*

We have identified a number of programs of excellence that are multidisciplinary, enfranchise the majority of our scientists, feature cutting edge science, and dovetail when possible with our clinical areas of excellence. Each program has developed a strategic plan that identifies short and long-term goals, potential for achieving national recognition, receiving unique funding and/or making significant breakthroughs or discoveries. They have also identified key resources that are deemed essential to realize the short and long-term goals. **Our goal is that five of our programs of excellence will receive national/international recognition over a five-year period.** Because of concerns about sequestration and further reductions in the NIH budget and the potential need to provide enhanced support for our investigators, we have allocated \$5 million per year to support this strategic plan. These funds are largely earmarked for recruitment, pilot grants, and special program support. An additional \$1 million per year has been allocated to support Bioinformatics, through the new Center for Biomedical Informatics.

Allocation of these funds among the programs of excellence will be prioritized by a “Scientific Advisory Council” (SAC) comprised of our top scientists and will be based upon the resources required, the potential for garnering extramural funding, and the potential for scientific breakthroughs or enhanced national/international recognition.

**Metrics:** Programs will be evaluated periodically to determine whether they are meeting their goals and merit additional SMD support. Whereas our previous strategic plans focused on growth of research programs and NIH funding, the focus of this plan will be on the quality and impact of research, and on raising the reputation of the medical center and university through the promotion of nationally and internationally recognized programs of excellence. Therefore, our key metrics will focus on such recognition. Standard bibliometrics, such as number of publications per year, or number of publications in high impact factor journals, do not adequately reflect quality or impact of research. However, a consistent history of impactful works by a research program can provide evidence of its contribution to a field – as reflected by article citations or other “consumption” metrics. Additional metrics and targets will include the following (not all of which may apply to each program): Measures of national (or international) recognition: Members inducted into the National Academy of Sciences, Institute of Medicine or similar institutions; Members being named leaders of a major national professional society or receiving a major national/international prize/award; or Receipt of a unique or very rare competitive grant/contract award (e.g., being named as one of only 3 national centers in a specific field). Discovery metrics: Creation of important new scientific knowledge with high impact. For example, identification of novel genes that individually or collectively regulate a specific phenotype disease. Such knowledge creation is often reflected by a series of inter-related high-impact publications that achieve national and international recognition. Translational metrics: Identification of new therapeutic targets; Preclinical identification of promising devices, diagnostics or therapeutics; patent filings; First-in-class approaches are especially impactful.. Clinical impact metrics: Practical applications, such as new devices/diagnostics or biologicals, as reflected by new INDs or IDEs, first-in-human trials; or licensed patents/products. Community benefit metrics: Research that produces measurable differences in quality of life, effectiveness of care or efficiency of care (cost), impact on care delivery, or lives saved. Policy metrics: Research that impacts on policies, laws or regulations, or leads to the establishment or improvement of federal, state or local health programs or statutes. Productivity metrics: A major goal of the strategic plan is to develop an infrastructure that helps promote the productivity of our faculty. This includes developing programs that are more competitive for NIH funding, more competitive for alternative funding (such as PCORI grants, industry support, etc) and that could generate significant intellectual property.

### Shared Challenges and Goals

In developing this strategic plan, a variety of shared challenges were evidenced. The decade-long decline in inflation-adjusted **NIH funding**, combined with the recent impact of sequestration, is a threat to all programs. Several programs are characterized by **physical separation** of many of their members. This results in a lack of programmatic cohesion and a suboptimal research environment. **Collaborations** among discovery **scientists and those who work with patient** populations potentially impacted by those discoveries are often not optimal. Most programs are actively using new technologies that generate large data sets, but lack the training, expertise, and scientific colleagues necessary to fully explore, integrate and leverage those data. There is an urgent need to expand institutional **biomedical informatics**.

The strategic planning process also identified a series of shared goals. **Training and mentoring** of the next generation of scientific leaders (students, postdoctoral trainees and faculty) is an essential goal for all programs. Financial **support for trainees** is critical to the success of the research mission, because trainees conduct a significant portion of the research in faculty labs. The establishment of partnerships with institutions in other countries, such as the UR China Institute, provides a pipeline of gifted trainees with at least partial support from non-

UR sources. Programs focused on early **career development** of faculty are essential, as are programs aimed at mid-career faculty with leadership potential. Flexible **sabbatical programs** are necessary to help established faculty “retool” as national research priorities shift, as are “**mini sabbatical**” programs that permit faculty and their trainees to undertake short (2-6 week) intensive training in new methodologies and techniques that will enhance their research. A robust **interim funding** program is essential to ensure that productive research teams can be maintained, even when there is a hiatus in extramural grant support. Several programs identified needs related to **drug repurposing and drug discovery**, including access to high-throughput screening and medicinal chemistry, and continued support for the SMD’s Drug Discovery Pilot Award program. The **Shared Resource Labs** within SMD (including the vivarium) are essential to the research success of all of the programs of excellence, and their continued support will be needed. **Scientific community building** was also stressed. This includes: 1) Seminar programs and program-oriented annual meetings/symposia; and 2) Space realignment and physical colocalization of faculty with aligned interests. **Pilot funding** is necessary to build new interdisciplinary scientific collaborations. Most programs identified a need for 1-2 pilot awards of \$50k each, per year. These funds should become an important philanthropic target.

There is a need to develop closer **ties to the private sector**, and to explore **non-traditional sources of funding**. This may require the development of appropriately aligned metrics within the Office of Technology Transfer (e.g., metrics that relate to the development of sponsored research programs and SBIR/STTR awards). All programs need to work effectively in partnership with **Advancement** to obtain resources necessary to achieve program goals. There is a particular need for support for endowed professorships and for funds that support scientific programs.

The following is a summary of the currently proposed programs of excellence. These programs bring together the vast majority of our scientists in new collaborative structures. We intend that this process will be dynamic and will allow groups of investigators to develop strategic plans for other programs throughout the next five years.

**A. Cancer (Hucky Land):** To advance personalized (precision) cancer treatment and to improve quality of life for cancer patients and survivors. The JP Wilmot Cancer Center has developed a strategic plan, revolving around four areas: **a) Hallmarks of Cancer/Biology** - Identify mechanisms underlying malignant cell transformation that are common to diverse cancers and can serve as targets for new therapeutics; **b) Hematological Malignancies** - Improve the clinical outcome in myeloid and lymphoid malignancies through novel therapeutic approaches; **c) Solid tumors** - Improve the outcome in gastrointestinal, genitourinary, breast and brain cancers by utilizing molecular diagnostics and targeting common cancer cell vulnerabilities; and **d) Cancer control and survivorship** - Patient-centered research with special emphasis on protecting cancer survivors from normal tissue damage and other adverse side effects of treatment.

**B. Immunity & Infection** (Steve Dewhurst, Tim Mosmann, John Treanor): To be a world leader in discovering how the immune system and microbes synergize to prevent or cause disease. There are 3 program goals: (1) To understand how the microbes that inhabit our bodies (our “microbiome”) influence our susceptibility to infection and autoimmune diseases. Each of us is “home” to literally trillions of microbes - which help to digest our food and protect us from pathogens, but may also determine our risk of allergy, obesity and autoimmunity. Information-driven technologies and computational analysis has revealed the interplay between the microbiome, the immune system and pathogens, leading to broad new insights into health and disease. (2) To understand how the immune system responds to infection, by directly visualizing immune cells within the body – rather than in the test tube. This is made possible by new

technologies to image sites of infection and the immune response in real time, creating important new knowledge that will inform the development of more effective vaccines and treatments. (3) To develop novel anti-microbial drugs and vaccines. Program members have identified pathogen-specific proteins and metabolic pathways that can serve as targets for new anti-infective agents, and are developing new treatments for deadly drug-resistant pathogens with the goal of initiating a first-in-human trial within 5 years.

**C. Musculoskeletal Diseases (Eddie Schwarz):** To be the world leader in musculoskeletal research that leads to novel diagnostics, treatments and standards of care. This program has three established areas of focus, for which we already have international recognition: **a) Orthopaedic Trauma** - Elucidate the mechanisms responsible for musculoskeletal degeneration and poor healing associated with injuries and aging. Develop novel therapies, including stem cell-based treatments, and systems of care to improve clinical outcomes and cost-effectiveness; **b) OsteoImmunology** - Elucidate the interactions between immune and skeletal cells to develop new approaches to treat inflammatory bone diseases; and **c) AOTrauma Clinical Priority Program**. This international program focuses on novel approaches (including the first product developed by our cGMP stem cell facility) to prevent and treat infection associated with bone injury and surgery.

**D. Cardiovascular Diseases (Charles Lowenstein):** Identify and target pathways of cardiovascular inflammation and fibrosis that cause atherosclerosis, heart failure, and sudden cardiac death. We have identified two programs that dovetail well with our clinical areas of excellence: **a. Heart Failure** - Identify molecular pathways that lead to heart failure, and develop new therapeutic approaches to prevent sudden death from heart failure; and **b) Vascular Inflammation and Fibrosis** - Elucidate mechanisms by which vascular inflammation and fibrosis cause atherosclerosis, ischemia, and other forms of cardiovascular disease.

**E. Systems, Cognitive, and Computational Neuroscience (Suzanne Haber, Greg DeAngelis).** To translate the latest systems neuroscience advances in understanding brain mechanisms into development of new therapeutic approaches and targets for treatment of neurological and psychiatric disorders. Research areas include: **a) Sensory Systems and Perception** - Translate the latest knowledge about how sensory information is represented and processed in the brain into novel approaches that harness the power of neural plasticity to restore or enhance perceptual functions; **b) Cognition and Decision-Making** - Apply new developments in cognitive neuroscience and “neuroeconomics” to better understand and treat disorders of self-control, language processing, and cognitive control; **c) Development and Aging** - Understand how cognitive functions develop in youth and decline during aging or disease; and **d) Motor Systems and Rehabilitation** - Understand how neural circuits in the brain and spinal cord orchestrate complex movements, and they can be utilized to facilitate recovery from strokes or to develop prosthetic limbs and brain-machine interfaces that can restore lost motor function.

**F: The Neuroscience of Neuroinflammation and Neurodegeneration (Handy Gelbard, Jonathan Mink):** To understand normal and pathologic immune function in the nervous system and to utilize this to develop new therapeutic strategies for major neurodegenerative diseases. Programs include: **a) Batten Disease Center of Excellence (awarded)** – Take a comprehensive approach to research, care and treatment of this rare, and ultimately terminal neurodegenerative disorder; **b) Cerebrovascular disease** - Identify the most promising molecular targets for small molecule therapies of strokes, global hypoxic-ischemic events, and traumatic injuries to the brain and spinal cord; **c) Neuroimmunology of Microbiome and Central Nervous System (CNS)**

**disorders** - Understand how perturbations in the normal gastrointestinal flora become altered by disease to induce neuroinflammation in the CNS; **d) HIV-1 and Multiple Sclerosis (MS)** - Understand mechanisms connecting HIV-1 associated neurocognitive disorders (HAND) and MS, and develop new small molecule therapies that can be aggressively advanced through preclinical studies to clinical studies; and **e) Development and Aging** – Elucidate mechanisms of CNS development, aging, and degeneration that will enable us to develop new treatments for neurodegenerative diseases. The UR is currently designated as a Center of Excellence by the National Parkinson Foundation and by the Huntington’s Disease Society of America.

**G: Neuromuscular Diseases** (Charles Thornton, Dick Moxley, Bob Holloway): The Neuromuscular Disease Center research group strives to identify the causes of and more effective treatments for the most prevalent forms of muscular dystrophy and inherited nerve diseases. Our research has focused particularly on myotonic dystrophy type 1 (DM1), the most common form of adult muscular dystrophy. Using a mouse model of DM1, we recently identified a treatment that reversed all abnormalities for over one year after twice weekly subcutaneous injections for only four weeks. With support from the NIH, industry, private foundations, and donors, we are preparing for initial trials of treatment in DM1 patients. If treatment proves successful in DM1 patients, it may lead to more effective treatment in more common diseases with similar manifestations.

**H: Center for Translational Neuromedicine** (Steve Goldman, Maiken Nedergaard): To understand neural stem cell and glial cell biology, and to use that understanding to develop new treatments for neurological diseases, using cell and gene therapy. This well funded and nationally-regarded program is designed to study the basic biology of glial and neuronal stem cells and to initiate clinical trials to treat a variety of neurologic diseases, including: **a) Pediatric leukodystrophies**, such as Pelizaeus-Merzbacher disease, Krabbe’s disease, Tay-Sachs disease, and mucopolysaccharidosis; **b) Multiple Sclerosis**; **c) Huntington’s Disease**; **d) Age-related white matter loss and vascular dementia**; and **e) Central nervous system cancers**, notably glioblastoma and oligodendroglioma. Several of these trials are likely to occur during the term of this strategic plan.

**I. Autism Research Center** (Loisa Bennetto, Susan Hyman, Tristram Smith, Christopher Stodgell, Alexander Paciorkowski): To create a premier clinical and translational research center focused on causes, characteristics, and treatments that will optimize the health and well being for individuals with an autism spectrum disorder (ASD). The UR has national prominence in all aspects of ASD research, which will be leveraged and enhanced by the establishment of an Autism Research Center (ARC). Programs will include: **a) Comparative Effectiveness Research**; **b) Health Services Research**; **c) Multisensory Processing and Communication**; **d) Comorbid Health Conditions**; **e) Genetics**; **f) Neurotoxicology**; and **g) Neurodevelopment**.

**J. Health and Wellness** (Nana Bennett): To be a national leader in prevention research in chronic and communicable disease. This program will include focus on two areas: **a) Chronic disease prevention**: This program targets the key determinants of health - tobacco cessation, physical activity, nutrition, stress management, cardiovascular disease risk reduction, and reduction of alcohol dependence. The approach includes the basic science of behavior change and implementation science addressing the translation of the basic science into model clinical and community programs. Known evidence-based programs (e.g., Diabetes Prevention Program) will be translated to community and clinical settings; and **b) Communicable disease prevention** - This builds on the Center for Community Health’s ongoing public health communicable

disease population surveillance program (funded by the CDC) for the prevention of healthcare-associated infections, and the study of the implementation and effectiveness of vaccines.

**K. RNA Biology** (Lynne Maquat, Dave Mathews): To apply cutting-edge basic science discoveries in RNA biology to clinically relevant translational research. The UR is internationally recognized as a leader in RNA biology, and Center research programs include: **a) RNA Structural and Computational Biology** - Analyses of RNA for the purposes of making and testing predictions about RNA function; **b) Therapeutics** - Development of new therapeutics that either target RNA or are themselves composed of modified RNA; and **c) RNomics** - Characterization of cellular transcripts and their associated protein binding partners. Targeting RNA regulatory processes or regulatory RNA molecules will provide powerful tools to designing novel therapeutic approaches for a broad range of diseases.

**L. Institute for Aging Research** (Vera Gorbunova, Dirk Bohmann, Yeates Conwell): Optimizing aging research to enhance health; optimizing health research to enhance aging. Basic research has matured to a stage where discoveries on fundamental cellular mechanisms of aging and longevity can be translated into testable hypotheses that can inform clinical research. A dedicated *Institute for Aging Research* will be established to facilitate interdisciplinary aging research across the institution. Elements of this program include: **a) Aging, Regeneration and Stem cells**; **b) HIV and Aging**; **c) Immune Responses and Vaccination in the Elderly**; **d) Oxidative Stress in Aging and Disease**; **e) Metabolism and Aging**; **f) Cancer and Aging**; **g) Alzheimer's Disease and Related Dementias**; and **h) Optimizing Care And Services for Older Adults**.

**M. Lung Biology** (Richard Phipps): To become a world leader in the study of lung health and disease. The UR has a strong environmental medicine and lung biology program that focuses on the following: **a) Environmental Lung Exposures Throughout Lifespan** - Understand the life-long effects of air pollutants and other respirable products; **b) Lung Infections** - How environmental toxicants influence the duration and type of infection; **c) Lung Inflammatory Diseases** - Study how stem cells contribute to lung scarring and how they can be used to rebuild damaged lungs; **d) Lung Cancer** - Research in this area is an important institutional research gap, which could be addressed by building on existing clinical and imaging expertise, as well as the current tobacco and biomass smoke program and the Cancer Center; **e) Cardio-Pulmonary Interface** - The effects of air pollution on blood platelets and on the heart and vasculature; and **f) Community Engagement & Public Health** - Integrate the nationally-recognized UR smoking cessation program and the scientists studying biomass and tobacco smoke.

**N. Comprehensive Diabetes Center** (Steve Hammes, Steve Wittlin, Victoria Hsiao): To create a Comprehensive Diabetes Center that focuses on the full spectrum of diabetes progression, from prevention to treatment of diabetes and its complications. Monroe County has approximately 70,000 individuals with diabetes and 80,000 individuals with pre-diabetes. It is paramount that URMC lead the way in developing effective and efficient ways to prevent and treat diabetes and its complications. The Division of Endocrinology has grown remarkably over the past 5 years, and was 21<sup>st</sup> ranked nationally in 2013 (US News and World Report). It is poised to lead the charge toward a Comprehensive Diabetes Center that would focus on: **a) Diabetes Complications**: Form a distinctive, bench-to-bedside research program in diabetes complications, with particular strength in the cardiovascular, neurologic and retinal complications of diabetes; **b) Diabetes Immunology and Autoimmunity** - Create a

multidisciplinary center focusing on autoimmunity and diabetes in adults and children; and c) **Islet Cell Biology** - This emerging field leverages strength in immunology and stem cell biology.

**O. Vision Restoration Program (David Williams, Steven Feldon):** To preserve, repair or replace damaged visual systems - thereby restoring sight to the blind and visually impaired. This will be achieved by leveraging existing strengths in optogenetics, optical engineering, neural plasticity, and clinical ophthalmology to develop breakthrough technologies for vision restoration and to translate them into clinical use. The program combines the Center for Visual Science (CVS), an internationally-recognized research center, and the David & Ilene Flaum Eye Institute (FEI), one of the fastest growing ophthalmology departments in the nation. Programs include: a) Vision restoration, b) Imaging the eye, c) Visual cortical function and plasticity, d) Vision correction and e) Human populations studies and clinical trials. An overarching objective is to develop retinal prostheses for restoring vision in the blind and visually impaired.

**P. Drug Targets/Mechanisms Program (Bill Tank, Alan Smrcka):** This program leverages expertise in studying the fundamental properties of **G-protein-coupled receptors (GPCRs), ion channels and cell adhesion molecules** to identify new drug targets and mechanisms by which these targets function, with the objective of discovering new therapies for many different diseases. GPCRs and ion channels are major targets for drug therapy. Drugs aimed at these targets generally possess high potency and selectivity, permitting the use of low drug dosages and minimizing side effects. We envision that these molecules will remain major drug targets in the future and plan to do cutting edge research to identify new GPCRs and ion channels to target and to investigate new mechanisms by which to target existing molecules and/or the signals that they modulate. We also envision that cell adhesion molecules will become major drug targets in the future. These molecules orchestrate how cells interact with each other in functional tissues. Identifying these molecules and understanding how they function should provide many new targets for drugs to either enhance or block cell communication.

**Q. Childrens' Research Institute (Nina Schor, Jill Halterman, Damian Krysan):** This program aims to become a world leader in the discovery of how organ systems and organisms develop in health and disease and in translation of those discoveries into prevention, treatment, and cure for disease throughout the life cycle. A trans-campus, trans-departmental enterprise, this program will bring expertise, both existing at UR and to be recruited, to bear on developmental biology and will establish the UR as a premier venue for interdisciplinary and translational research and training that fuels the therapeutic and preventive armamentarium for generations to come.

**R. Environmental Medicine (Tom Gasiewicz, Deborah Cory-Slechta, Paige Lawrence):** This program seeks to prevent disease and improve public health through the integration of basic science and clinical research, and elaboration of mechanisms by which environmental exposures, alone or through interactions with other modifying factors, influence human health across the lifespan. The program focuses on: **a) Prevention and Treatment of Developmental Disease** - understanding how environmental exposures influence developmental disorders such as ADHD, asthma and autism; **b) Immunity and Inflammation** - defining how chemical and physical agents cause tissue injury and alter repair processes; **c) Impact of Early Exposures Enhance Susceptibility to Disease Later in Life** – including air pollutants, maternal exposures and developmental obesogens; **d) Stem Cells and Epigenetic Pathways as Targets of Xenobiotic Exposure;** **e) Nutritional and Dietary Factors** – as modifiers of environmental disease, and

possible interventions; **f) Environmental Epidemiology** – evaluating effects of environmental contaminants in exposed populations.

**S. HIV/AIDS Research** (Steve Dewhurst, Handy Gelbard, Mike Keefer, Amneris Luque, Dave Mathews, Jim McMahon): This program seeks to improve the prevention, detection and treatment of HIV infection, AIDS and related disease processes. The program focuses on: **a) HIV CNS Reservoirs and Aging** - understanding the interaction of HIV-1 with the CNS, exploring the interplay between frailty, aging, cerebrovascular function and cognitive decline, and developing new therapeutics; **b) HIV RNA Biology** - studying the role of RNA structures in HIV replication and pathogenesis, and developing new therapeutic strategies for targeting HIV at the RNA level; **c) HIV, Trauma and Disparities** - understanding how trauma, interpersonal and structural violence, and mental health impact HIV risk behavior and health outcomes, with a focus on disparities and the physiologic impact of trauma and stress; **d) Novel Vaccines and Therapeutics** - developing and testing novel therapeutics and vaccines relevant to HIV/AIDS and related diseases.

## **2. TO IDENTIFY KEY CORE RESOURCES THAT WILL BE NECESSARY TO PROMOTE THESE PROGRAMS OF EXCELLENCE AND ASSURE THAT WE CAN PERFORM STATE-OF-THE-ART CUTTING-EDGE SCIENCE.**

A small set of key, core resources have been identified as essential to supporting our programs of excellence. These include biomedical informatics, stem cell biology, and imaging. It is envisioned that these key resources will in themselves be centers of scientific excellence, in addition to providing necessary cores for the entire research enterprise.

**A. Center for Biomedical Informatics (CBI)** (Dirk Bohmann, Hucky Land, Rob Strawderman, Karl Kieburz, Martin Zand, Tim Dye). New research technologies are resulting in the generation of unprecedented amounts of data, at decreasing cost. As a result, the analysis and interpretation of large amounts of scientific information (“Big Data”) is increasingly becoming the major bottleneck in biomedical research. At the same time, changes in healthcare are resulting in the near-universal utilization of electronic medical record systems that are populated with enormous and highly diverse datasets that present new research opportunities and challenges. Our highest priority will therefore be to establish the **Center for Biomedical Informatics (CBI)**; \$1 million per year has been earmarked for this initiative.

The Center will develop and enhance the URM’s research programs in biomedical informatics, across the domains of basic, translational, clinical and population-based biomedical research. The Center will serve as the academic home and campus hub for researchers who employ informatics as a primary tool in their biomedical research activities, and will also support new educational programs in the area of biomedical informatics. There is also the opportunity for synergy with URM’s clinical programs, through the secondary use of clinical data for research purposes, and by collaborating on clinical care improvement initiatives that are founded on the aggregation and analysis of big data.

The CBI will comprise multiple interdisciplinary teams organized in two complementary divisions, addressing basic and translational research (Experimental Bioinformatics) and clinical, outcomes and population health research (Clinical Research Informatics). These teams will share databases and tools, and will collaborate on new methods development, and educational programs. Key activities will include:

- **Research:** The CBI will encompass the domains of population health research, clinical research, translational research and basic research.

- **Education:** The CBI will, in collaboration with the CTSI, train the next generation of scientists and physicians in biomedical informatics.
- **Infrastructure:** The CBI will, in collaboration with the Center for Integrated Research Computing (CIRC) establish the informatics infrastructure necessary for basic research and will collaborate with the CTSI to establish the informatics infrastructure necessary for clinical and translational research.
- **Research support services:** The CBI will facilitate and help to coordinate comprehensive biomedical informatics support services that address faculty research needs. The CBI will collaborate with CIRC to address the needs of basic research and will collaborate with the CTSI to address needs in clinical and translational research.

**B. Rochester Center for Health Informatics (RCHI) (Martin Zand).** The mission of the RCHI is to build the “learning healthcare system”, which will use healthcare data and cutting-edge analytics to improve the health of our community – with the goal of making Rochester the healthiest community in the nation. The learning healthcare system is a laboratory to iteratively improve population health and healthcare in partnership with the community. It will assemble and analyze massive amounts of data to study the anticipated and/or realized effects associated with transformations in population health and healthcare delivery and financing.

The RCHI mission will focus on specific projects related to population health, clinical care management and delivery. A secondary goal will be integration of basic science data and new data science analytic methods into these areas of research. The RCHI’s activities will include: (1) data aggregation; (2) data analytics; (3) modeling of health policy or delivery changes; (4) field-testing of interventions; (5) policy development and (6) best practices implementation. The RCHI will collaborate closely with strategic partners outside the University (including community organizations) to identify and implement new research collaborations, and will assist the Institute for Data Science in finding applications for new analytic methods across the Medical Center.

**C. UR Stem Cell and Regenerative Medicine Institute (SCRMI) (Mark Noble).** The SCRMI functions as an independent scientific program of excellence, and also provides essential infrastructure and organizational support for stem cell research. Specific research goals include the following: a) Develop treatments for multiple neurological and musculoskeletal diseases; b) Develop novel interventions to protect stem/progenitor cell populations in diseases of development and aging; and c) Discover new cancer therapies by exploiting differences between normal stem/progenitor cells and tumor cells. The Institute also provides essential support for bringing discoveries in stem cell biology to the clinic. The current Upstate Stem Cell cGMP Facility supports production of stem cell products under FDA-mandated guidelines, and is funded by NYSTEM as well as ongoing support from the URMC.

Important goals of the SCRMI include providing pilot program support for innovative research, and creating a new **Upstate New York Stem Cell Analytical Facility (UNYSCAF)** intended to (1) accelerate the development of scaled-up, FDA-compliant cell production processes, thereby accelerating new, first-in-human trials of stem cell therapies at UR and (2) identify pharmacologic and environmental agents that inhibit stem and progenitor cell function (and thus could reduce the likelihood of successful of clinical trials in stem cell medicine).

**D. Imaging Core (Suzanne Haber):** This core will develop and maintain “state of the art” large animal and human magnetic resonance imaging (MRI) facilities. It will leverage the infrastructure provided by the Rochester Center for Brain Imaging (RCBI), and will add new instrumentation to better serve the needs of a large and growing cohort of basic and clinical

investigators who use MRI imaging methods to study aging, neuromedicine and musculoskeletal disease in large animal models and in humans. The core will also provide pilot funds, as well as custom hardware and software, and will contribute to training of young investigators.

**E. Information Technology Support for Research (Steve Dewhurst):** The current SMD Academic IT unit will be restructured in order to better address the IT needs of the school's research mission. Key goals include providing research faculty with greater input into IT-related initiatives, developing a more pro-active approach to the development and support of data services for research, and ensuring that we are better positioned to work with research data that contain protected health information.

### **3. TO IMPLEMENT A RESEARCH INFRASTRUCTURE BEST SUITED TO ADVANCING OUR RESEARCH PRIORITIES.**

Research at URMC is currently conducted in 34 Departments and Centers. This necessitates duplication of some services and infrastructure. The proposed focus on collaborative programs of excellence that cross many departments and centers may provide opportunities for efficiencies that reduce costs. To address these issues, to help prioritize the programs of excellence, and to help manage central funds for research, we have established a new Vice Dean for Research and a Scientific Advisory Council (SAC).

**A. Vice Dean for Research (Steve Dewhurst).** The vice dean will report to the dean of SMD and will be responsible for: **a) Strategic Planning and Plan Implementation** - 1) Chair SAC; 2) Oversee SMD research strategic plan development and implementation; 3) Oversee research space planning; 4) Facilitate development of the CBI; 5) Oversee integration of training with research; and 6) Facilitate engagement of the advancement program with SMD research faculty; **b) New Collaborations and Partnerships that Diversify Research Funding** - 1) Facilitate partnerships with industry (including sponsored research and SBIR/STTR awards); 2) Act as tech commercialization and OTT liaison; 3) Facilitate big awards and new funding streams, including major federal center and contracts, and large foundation awards; and 4) Oversee the stem cell GMP facility; and **c) Other Responsibilities** - 1) Facilitate communication of faculty concerns to leadership; 2) Assist with faculty retention issues; 3) Assist with chair/center director reviews, when appropriate; and 4) Advise on allocation of core budgets for research.

**B. Scientific Advisory Council (SAC).** SAC will advise the dean and vice dean on strategic decisions relating to recruitment, academics, infrastructure investments, and space allocations. SAC will be chaired by the vice dean, and will include senior faculty members from the basic and clinical research communities, spanning a broad range of disciplines and focus areas. It will provide high-level guidance, to identify areas of need, and to provide advice on prioritization of projects/recruitments and areas for investment; it is not expected to review or approve the qualifications of individual faculty candidates. SAC will also administer and award a new "incubator" program for innovative, trans-disciplinary science.

**C. Clinical and Translational Science Institute (CTSI) (Karl Kiebertz).** The Rochester CTSI provides a robust academic home for clinical and translational research, and uses its resources to further the URMC research strategic plan and to meet the national CTSA program goals. It supports a small number of targeted, 'break through' research activities at the URMC, by identifying common 'sticking points' or 'stumbling blocks' in the research enterprise. Removing these impediments should permit new projects to 'break through' to productivity and independence. Although the CTSI will help catalyze a broad range of transitions, there will be a special emphasis on therapeutic development - including stem cell based therapies, biologicals

such as vaccines and small molecule approaches for CNS diseases. Resources necessary to enable the CTSI's mission include: 1) a **director of research bioinformatics**; 2) a small bore MR instrument for animals and upgrading of the research MR capabilities (see the imaging core); 3) a **clinical trial statistician** with an interest in early phase trials and adaptive trial designs; 4) a **cell therapy and biologics regulatory specialist** with experience in regulatory submissions to the FDA; and 5) additional **expertise in health economics**. Funding for some of these positions is already available as part of the institutional support for the CTSI, and the departments of Public Health Sciences and Biostatistics and Computational Biology.

## 4. PROGRAM PRIORITIZATION AND PLANS FOR FY15

### Principles for Prioritization of Research Programs

Prioritization will be flexible and responsive to emerging needs & new opportunities. Thus, priorities will be revisited annually. Factors that will drive resource prioritization will include:

- ✓ Critical mass and current strength of program: Programs must build on strength & serve needs of a broad number of faculty.
- ✓ “Bang for the buck”: Programs must articulate a vision that can be realized with specific resources; return on investment will be a major consideration for resource allocation.
- ✓ Leveraging other investments: For example, partnering w. the Institute for Data Science.
- ✓ Fundraising success: Programs that can successfully fundraise will achieve priority, as will those that can bring in non-SMD funds.
- ✓ Accountability: Programs will be accountable for meeting self-defined metrics; if these are not met, future investment will be curtailed.

### Priorities for FY15

Priorities for FY15 will include the following:

- ✓ Support existing faculty. Protect departmental budgets, most of which go to faculty salaries; generate endowed chairs.
- ✓ Honor existing commitments (open searches). Examples include active searches in RNA Biology and Immunity & Infection.
- ✓ Recruit in areas of broad institutional need. Recruitments into the Center for Biomedical Informatics; hiring of the Del Monte Neuromedicine Institute Director.
- ✓ Place emphasis on junior recruits. Where possible, recruit starting Assistant Professors to ensure that we infuse “young blood” into our faculty.
- ✓ Ensure scientific vitality of our current research programs. Make targeted investment to maintain a critical mass of researchers in departments/centers where projected retirements may substantially reduce the size of the research faculty.
- ✓ Support new programs/collaborations through SAC. Fund the new SAC incubator program, and new “program-building” pilot awards in broadly impactful areas – (i) Aging (RoAR), (ii) Prevention, (iii) Stem Cells & Regenerative Medicine, (iv) HIV/AIDS. Also, cofund multidisciplinary University Research Awards to SMD faculty.
- ✓ Provide pilot support in other strategic areas. Continue to support the Drug Development Pilot Award program. Strengthen the Technology Development Fund (TDF).