Proposal to Establish
The Ohio State University
Gene Therapy Institute

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I. Introduction

Critical molecular biologic insights have led to the fundamental understanding of the underlying genomic mechanisms of disease. These findings have led to the identification of therapeutic genes that can be packaged in viral capsids for the treatment of a variety of conditions, including neurodegenerative, hematologic, oncologic, muscular, metabolic and enzyme deficiency disorders. Data demonstrate that gene-carrying viral vectors (most often adeno-associated viruses [AAVs]) can effectively promote transgene expression in target tissues and cells. For systemic disorders, intravascular infusion of vectors carrying therapeutic genes is frequently used and can have wide spread impact. Nervous system disorders are most commonly treated by direct intraparenchymal infusion (convection-enhanced delivery), which bypasses the blood-brain barrier in a targeted manner and distribution can be monitored using real-time magnetic resonance (MR)-imaging. Moreover, the unique properties of viral vectors permit for the cell-specific transgene manipulation of the infused anatomic site and/or widespread interconnected sites via antero- and/or retrograde transport in the nervous system. These gene therapy paradigms have led to the successful treatment of incompletely or untreatable disorders.

Taken together, the implementation of therapeutic gene targeting in disease has required the robust implementation of collaborative expert University-wide teams of faculty. These faculty have enabled the successful translation of basic science discoveries into new therapies that are accessible to affected populations around the world. The formation of The Ohio State University Gene Therapy Institute will serve to define and support the efforts of these faculty and trainees that arise from diverse academic backgrounds, including, but not limited to the Colleges of Medicine, Arts and Sciences, Law, Business, Veterinary Medicine, Engineering and Pharmacy. It is the experience and expertise that exists at The Ohio State University that make it a global leader in gene therapy. While The Ohio State University is a pioneer in gene therapy applications, the rapid expansion and use of gene therapy globally make it critical to organize, structure and strengthen efforts in this the fast-moving field, to maintain our differentiating stature. Specifically, the faculty involved in these efforts have recognized this need and have underscored the importance of forming an institute to further support critical administrative, recruiting and infrastructural needs in the most effective and efficient manner.

While this initiative could reside as a College of Medicine or Wexner Medical Center institute, we strongly believe that the depth and breadth of talent, educational programs, partnerships and technologies across the entire university necessitate this structure as a university institute to both accelerate and accentuate the impact of this work for research, educational and clinical impact.

Institution Positioning

The Ohio State University intramural faculty involved in gene therapy have uniquely positioned the institution as a global leader in this area, with end-to-end capabilities ranging from fundamental science to clinical trials and, ultimately, commercialization. Successful and efficient translation of gene therapeutics into new patient treatments is a complex process that requires expertise from several domains and the continuum of development to be successful. Broadly, these domains include fundamental research (e.g., disease genomics, plasmid development, engineering delivery devices), product development/quality control (e.g., vector production/assessment and device assessment), clinical trial management, regulatory expertise, academic-private collaborations, management of intellectual property and operational excellence. The Ohio State University Gene Therapy Institute has expert member faculty representing the breadth of these areas. **Box 1** summarizes some of the differentiating features based on collaborations across faculty. Establishing The Ohio State University Gene Therapy Institute will augment and grow these differentiators.

Box 1.
Ohio State University Gene Therapy Expertise

- Over 50 participating faculty from 6 colleges.
- Largest first-in-human clinical trial portfolio internationally, with 10 active trials.
- Emerging research and trial platforms in neurologic and non-neurologic areas.
- Multi-million dollar grant support (including $14.6M National Institutes of Health [NIH] grants for a first-in-human nervous system gene therapy trial and a $4.8M translational grant for CRISPR-gene therapy research).
- On-site viral vector development and production capabilities.
- World-renowned expertise in nervous system drug drug delivery and viral vector development.
- Gene therapy-related research collaborations with industry partners, including Battelle, Medtronic, Biogen, Voyager, uniQure and Bayer.

Gene Therapy Portfolio

NIH investment into gene therapy research has doubled over the past decade. Funding grew from $6.2B across 15,238 projects in 2011 to $13.2B across 25,788 projects in 2020. The greatest growth in NIH investment for gene therapy research has been in the neurosciences. Funding has over doubled in the last decade from $438M across 1,367 projects (2011) to $1.1B across 2,239 projects in 2020[1]. Similar trends also exist for project funding by the Veterans Administration and Food and Drug Administration. The funding landscape for gene-based therapeutics, both in central nervous system disorders and in health research in general, is broad

[1] "NIH RePORTER." *National Institutes of Health*, U.S. Department of Health and Human Services, 2022, https://reporter.nih.gov/search.

and maintains an exponential trajectory. Thus, it is expected that the proposed work within this institute will attract significant extramural federal funding for the foreseeable future. Further, according to Janet Lambert, CEO of the Alliance for Regenerative Medicine, "In 2020, Gene and gene-modified cell therapy financing increased by 73% at $17.3 billion."[2] This will attract further potent financial support from foundation and industry partners. This makes gene therapy one of the most (if not the most) fundable areas in the life sciences.

To exploit the advantages of gene therapy, investigations are ongoing that will answer the key questions related to safety, route of delivery, plasmid/vector development and targeting. While gene therapy for non-neural disorders is typically treated using systemic administration (i.e., intravascular), nervous system-related disorders have added complexity to treatment related to the blood-brain barrier (natural neurovascular barrier) that is a significant impediment to gene therapy delivery from the system circulation.

To overcome this barrier, faculty at Ohio State have pioneered the use of direct intraparenchymal delivery (convection-enhanced delivery) to perfuse targeted regions in the brain to distribute viral vectors. This technique permits the infusion of viral vectors for gene therapy and is a safe, homogeneous and reliable manner that bypasses the blood brain-barrier. Moreover, real-time MR-imaging can be used to accurately assess vector distribution in real-time during delivery using intraoperative MR-scanning.

The unique properties of convection-enhanced delivery of viral vectors permit for cell-specific transgene manipulation of the infused anatomic site and/or widespread interconnected sites via antero- and/or retrograde transport. Nearly half of all gene therapy clinical trials use direct convective delivery as their delivery platform (**Figure 1**).[3] The use of convective delivery in the nervous system for gene and non-gene therapies was pioneered by faculty (Lonser and colleagues) at Ohio State.

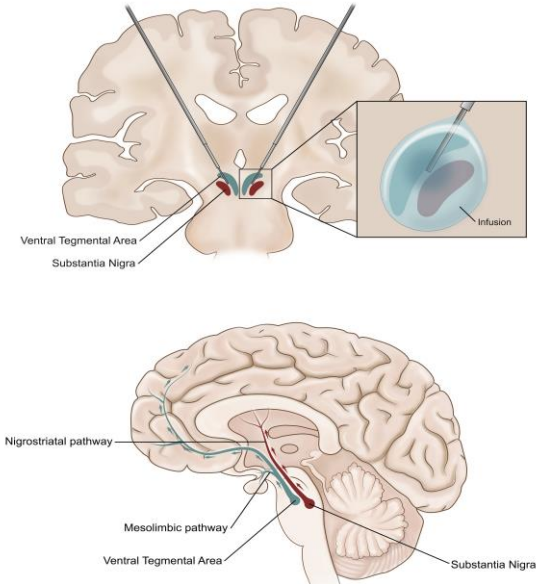


Figure 1. Illustration demonstrating direct convective delivery of gene therapy for manipulation of neurociruitry in aromatic L-amino acid decarboxylase (AADC) deficiency disease using adeno-associated viral (AAV) vector containing the AADC gene. (*Top; coronal view*) Anatomic treatment targets for treatment include the substantia nigra and ventral tegmental areas, which are critical dopamine producing areas (via conversion of L-Dopa to dopamine). (*Inset*) These anatomic structures are perfused with AAV-AADC (*blue*) to replace deficient AADC. (*Bottom; sagittal view*) Because AADC deficient patients have intact nigrostriatal and mesolimibic pathways, perfusion of the substantia nigra and ventral tegmental area have potent distant effects via anterograde (*arrows*) transport in these pathways.

[2] Alliance for Regenerative Medicine's annual Cell and Gene State of the Industry Briefing, 2022
[3] R.R. Lonser et al., Direct convective delivery of adeno-associated virus gene therapy for treatment of neurological disorders. ***J Neurosurg*** 134:1751-1763, 2020.

Based on the early discovery and the defined properties of adeno-associated viral (AAV) vectors, they are historically the most characterized vectors for gene therapy. Ninety percent of gene therapy trials use AAV vectors for transgene expression in neurologic and non-neurologic disorders. Advantages for the use of AAV capsids, compared to other vectors, for gene therapy include: they are small, non-replicative, can transfect dividing/non-dividing cells, provide lasting/permanent gene expression and they are not pathogenic in humans.

Faculty (Bankiewicz and colleagues) at Ohio State have been pioneers in the AAV gene therapy research field and continue to innovate and advance our understanding of the safety and optimal delivery methods for early AAV serotypes, as well as emerging AAV variants. They are currently characterizing new AAV serotypes with favorable characteristics for disease-specific treatments. Specifically, this group has built an AAV vector producing core for research purposes that is being developed to meet good laboratory and good manufacturing practice standards. Work from the laboratory of Arthur Burghes in the Department of Molecular Genetics is developing strategies that allow AAV to be used for gene therapy of Duchenne muscular Dystrophy.

Based on the expertise and infrastructure at Ohio State, there is a unique foundational platform that provides opportunity to be *the* world leader in gene therapy. To advance this opportunity and exploit emerging prospects across the gene therapy development continuum, Ohio State will need to include expertise across multiple colleges in a more comprehensive and complete way. To do so, it will be critical to develop a formalized structure and effective support through a Gene Therapy Institute. The institute will provide a cohesive environment for university faculty to tackle the unique clinical challenges of gene therapy research (basic, translational and clinical), vector development/production, device/delivery engineering, operational efficiency, physician training and innovative business models to ensure maximal patient benefit. This requires an innovative, enterprise-wide Institute.

Defined Areas of Impact

There are a number of critical points of institutional opportunity over the continuum of gene therapy development that would be derived from a Gene Therapy Institute. These opportunities span research discovery, vector development/production, clinical trials, regulatory assessment, operational efficiency and training, as defined below.

Foundational Disease-Modifying Gene Therapy Research. Disease-modifying basic science discoveries by Ohio State faculty and extramural partners will provide a critical catalyst for reducing the burden of disease across the spectrum pathology. The listed intramural faculty scientists are at various stages of gene therapeutic development across diseases from all biologic systems. These early discoveries will be most effectively translated into transformative clinical modalities through the defined administrative, infrastructural and collaborative expertise

of the Gene Therapy Institute. Moreover, the expansion of these novel disease-modifying programs has led to the recruitment of leading scientists from around the world.

Plasmid and Vector Development. To further enhance gene therapeutics, it is important to evolve plasmid and vector design. Current viral vectors will be replaced by enhanced vectors (e.g., those that target specific cell populations and/or employ cell-to-cell directed transmission) and other non-viral vector technology. Consequently, it will be essential to continue to support and recruit teams of scientists that work in this research area. Moreover, it will be critical to continue to pursue regulatable gene therapy constructs to tune and control treatment effect most precisely in some disease states. The Institute structure will facilitate the rapid and efficient development and implementation of these constructs across basic, translational and clinical research streams.

Vector Production. Ohio State has pioneered the capability to successfully produce AAV for gene therapy research. AAV is the most common vector used in gene therapy research and clinical trials. Using the infrastructure of the Institute, vector production could be developed to meet the Good Laboratory Practice (GLP) and/or Good Manufacturing Production (GMP) standards. On-site vector production would then not only support the research needs of our faculty, but provide a significant revenue stream via the sale of vector to outside research and commercial interests. The University-wide faculty, including those from the Colleges of Law and Business, provide critical expertise in operations, intellectual property, technology transfer and commercial/regulatory development of this program.

Clinical Trials. Ohio State is a recognized international leader in first-in-human gene therapy clinical trials (particularly, nervous system gene therapy). These include both investigator-initiated (by Ohio State faculty) or industry-initiated gene therapy clinical trials. Investigator-initiated trials are supported by foundation/federal funding, which will contribute to sustaining the activities of the Gene Therapy Institute. In addition, Ohio State's international leadership in critical areas of gene therapy has led to academic-industry partnerships that drive significant resources from running these trials that not only support research, but also provide revenue to The Ohio State University Wexner Medical Center). For example, a recent Huntington's disease gene therapy trial provided a net margin of $1.4 million (mean net margin per patient, $103,000) over the last 1.5 years. The clinical expertise and trials have made The Ohio State University Wexner Medical Center an international medical destination. Over the last 2 years, 76% of nervous system gene therapy patients came from outside the state of Ohio or from outside of the United States. The Gene Therapy Institute would catalyze this type of clinical activity.

Device Development. Device development will be critical to deliver gene therapy successfully and most effectively. Teams of physician-scientists and engineers at Ohio State are developing devices to more effectively and efficiently deliver gene therapy to targeted areas of the nervous

system (as vector does not cross the blood-brain barrier). Moreover, scientists at Ohio State are working with industry partners to enhance imaging of gene therapy distribution to improve safety, enhance treatment effect and assess clinical impact. The early effect of these research developments has led to investigator/institutional patents, potential new investigator/institutional companies and funding (including equipment infrastructure) streams from industry partners. The Gene Therapy Institute would provide a structure and focus for these activities, lowering the barrier to interdisciplinary collaborations with device development researchers and providing focused platforms for device development addressing critical gene therapy needs.

Strategic Partnerships. Currently, Ohio State has developed and is developing critical academic-industry partnerships in gene therapy research and development (see **Box 1**). These relationships provide significant and defined research funding streams that can drive faculty academic aspirations and related basic, translational and clinical research programs. These partnerships have offered access to state-of-the-art industry-based research programs (e.g., pre-clinical platforms launched as first-in-human clinical trials at the Ohio State University). Moreover, based on the Ohio State University expertise in this area, industry partners are providing significant support to manage pre-clinical and clinical gene therapy portfolios, which has substantial academic career benefit to our involved faculty. Finally, this institutional expertise could be developed into an Ohio State University Gene Therapy Institute contract research organization to provide additional revenue. The proposed Gene Therapy Institute would provide a focal point in the university for developing strategic industry partnerships, and would offer industries an end-to-end cadre of researcher expertise and capabilities that industries seek

Education. Education is a critical component of the Gene Therapy Institute. Workshops, networking and didactic events will be scheduled on a regular basis (see below). Additionally, areas of gene therapy will require defined hands-on training to effectively and safely treat patients. Specifically, because it is a new field, direct delivery of gene therapy in nervous system will require specialized training. Ohio State is the global leader in this technology and is poised to train other neurosurgeons from around the world. This training component will be a lasting potent institutional differentiator, provide an effective training-associated revenue stream and serve as an incubator for the development of new delivery technology. Finally, this field, like many others emerging in Ohio (Intel, Amgen, etc.) will require a talented workforce with experience in technology, bioinformatics, artificial intelligence, education, law, regulatory, business, etc.). Thus, we view this area as a critical leader in future training for undergraduate, graduate and faculty at Ohio State.

Developed Patterns of Operational Excellence. Critical to each of the areas described previously is operational excellence. Associated operational excellence in gene therapy has unique components that include vector production, intellectual property/technology transfer,

academic-industry partnerships, clinical trial design, regulatory monitoring, new company development, training and facility management. Institute faculty from the Colleges of Law and Business are experience experts in these areas. Based on their expert input and program development, the Gene Therapy Institute will be a leader in the effective operational and systems management across all platforms. These platforms will serve as institutional differentiators across all our college faculty collaborators.

Product Licensing and Company Development. The expertise and research findings derived from the Gene Therapy Institute faculty will drive critical discoveries and intellectual property in this area. This has been an excellent source of financial sustainability resulting from licensing fees, new company formation and other product development opportunities at academic institutions around the world.

Recruitment. Based on its excellence and institutional differentiating features, the Gene Therapy Institute programs will be a foundational component to recruiting faculty across all the associated Colleges. We have already begun to see the impact on recruitment of international leaders in the related areas defined above. The formation of an Ohio State University Gene Therapy Institute will further underscore, support and drive acquisition of the best worldwide talent.

II. Mission

Missions of the University

> *"For anyone familiar with the slow crawl of drug development, gene therapy is moving at lightning speed. The approval of the first two gene therapies in the US and promising clinical data on many more have supercharged the field."* ***Chemical Engineering News***, September 2019

A university-wide plan has been established in consultation with stakeholders from various colleges interested in the advancement of the education, technology and medical opportunities in gene therapeutics. There was consensus that to become and maintain a global leadership role in this area that an Institute structure was required. The Ohio State University Gene Therapy Institute has the following mission:

- Build an interdisciplinary community in gene therapy science and transformative care.
- Support the infrastructure to enable investigators rapid access to cutting edge technology in gene therapy vectors and delivery devices.
- Develop a new business model for The Ohio State University.
- Lead in the education and training of gene therapy in the areas of science, law and business.

Moreover, the mission of The Ohio State University Gene Therapy Institute aligns with the Strategic plan for the Office of Research:

- Build and sustain a culture of excellence that attracts, engages and supports top faculty and staff talent, enabling them to perform at the highest levels.
- Attract, develop and support an inclusive and innovative community to educate future research leaders who create impact in academia, industry and broader communities.
- Establish The Ohio State University as the leading university in interdisciplinary research and creative expression.
- Provide high quality, innovative physical space, infrastructure and financial support for research and creative expression.
- Broadly expand The Ohio State University's research and creative expression engagement beyond the campus to accelerate impact.

Below, we summarize some of the specific aspects of research and education that form the foundation of The Ohio State University Gene Therapy Institute mission and the defined areas of impact that advance the strategic plan of the Institute and University.

Defined Strategies

Research. The Ohio State University Gene Therapy Institute will support current faculty and continue to recruit research leaders. The Institute will provide an infrastructure and leadership focused on operational excellence. Recruitment and retention strategies will focus on inclusion and diversity to foster a culture of excellence and innovation (see additional details in the section below). To accomplish these goals, we will do the following:

- Build a community to facilitate collaborations for critical internal and external stakeholders. A robust collaborative environment will enable investigators to obtain support for translational research projects.
- Maintain a viral vector production core for rapid progression in discovery science.

- Establish and maintain a small and large animal research core for the characterization of viral distribution, safety and efficacy.
- Support internal quarterly topic-focused research in progress meetings and an Annual Institute Retreat.
- Support expansion of first in human clinical trials, provide dedicated clinical facility and faculty, regulatory expertise and enhanced industry partnerships.
- Support critical core facilities that include: vector, therapeutic discovery/development, animal, device development, regulatory, clinical trials and product development cores (**Box 2**).

Box 2.
The Ohio State University Gene Therapy Core Facilities

- Vector Production
- Therapeutic Discovery/Development
- Animal Studies
- Device Development
- Regulatory
- Clinical Trials
- Product Development

Recruitment. The Ohio State University is committed to establishing a culturally and intellectually diverse environment, encouraging all members of our learning community to reach their full potential. We are responsive to dual- career families and strongly promote work-life balance to support our community members through a suite of institutionalized policies. To build a diverse workforce, The Ohio State University encourages applications from individuals with disabilities, minorities, veterans and women. Ohio State is an Equal Opportunity/Affirmative Action Employer. All qualified applicants will receive consideration for employment without regard to race, color, religion, sex, sexual orientation, gender identity, national origin, disability status or protected veteran status.

Education and Training. Didactic and experiential education of faculty, trainees and staff are at the center of the Gene Therapy Institute mission. The diverse set of learners and the cross-discipline set of educators provides a unique learning environment that will drive quality and innovation. To achieve the education and training goals of the Institute, we will do the following:

- Establish targeted early career training and education that is learner specific.
- Provide laboratory training opportunities in faculty laboratories, post-baccalaureate, PhD/MD/PhD students, clinical residents, fellows, junior faculty.
- Provide consultation and training for faculty interested in clinical trial design and implementation.
- Establish internship programs with intramural investigators and/or extramural partners (research, business and law).
- Develop networks for career development.
- Organize workshops for internal and external collaborators.
- Provide programs for specialized gene therapy clinical training for physicians and surgeons in areas of need.

Opportunities to Collaborate across Ohio State

Synergy with existing institutional research programs is critical. It is important to note that the GTI will serve as a force multiplier within the gene therapy space and synergize with multiple existing programs to add additional value.

Neurological Institute. The Neurological Institute was designed to enhance the quality and breadth of neurological-related research at Ohio State by providing an infrastructure with an emphasis on translating research discoveries into clinical interventions. Comprehensive resources include an integrative brain bank and biorepository, mentorship and career development opportunities for junior investigators and world-class educational symposiums. To meet this goal, the Neurological Institute prioritizes facilitating collaborations between clinicians and scientists, fostering educational and professional development activities for junior investigators in the field and building a world-class infrastructure to support translational neuroscience research.

Critical opportunity. The broad membership of the Neurological Institute provides a strong foundation and opportunities for synergy with the Gene Therapy Institute. For example, researchers from the Neurological Institute will be exposed to new gene therapy related projects and resources, facilitating new collaborations and a potential bridge between gene therapy researchers and neuroscientists from other disciplines to bridge critical gaps in knowledge. In addition, the education and mentorship platforms currently in place at the Neurological Institute can be augmented by content specific expertise related to gene therapy.

The Steve and Cindy Rasmussen Institute for Genomic Medicine. The Steve and Cindy Rasmussen Institute for Genomic Medicine at Nationwide Children's Hospital was created to enhance pediatric personalized genomic medicine. The Institute for Genomic Medicine combines a robust clinical laboratory with genome scientists and clinical geneticists. Collaboration among clinicians, physician-scientists, and basic science investigators is emphasized to quickly transition novel research results into advanced diagnostics, using state-of-the-art technology. Key branches of the Institute for Genomic Medicine include the clinical laboratory, the genomics services laboratory and technology development. The Institute for Genomic Medicine clinical laboratory performs high complexity molecular genetic analysis, cytogenetic analysis and advanced genomic testing. The Institute for Genomic Medicine also provides next-generation sequencing through the genomics services laboratory, which assists investigators with multiple aspects of next-generation sequencing, including experimental design, library preparation, sequence generation and data analysis. The Institute for Genomic Medicine Computational Genomics Group comprises a dynamic team of 10 computational biologists with the substantial technical and bioinformatics expertise required to oversee the multiple platforms that acquire, store and analyze large and complex data sets generated by the Institute for Genomic Medicine genomic services and technology development laboratories. The

technology development team focuses on translational approaches with new sequencing technologies and methodologies, as well as supports the mission to provide high quality genomics as a critical clinical resource.

Critical opportunity. The science of gene therapy is rooted in genomic medicine and the expertise and resources of the Institute for Genomic Medicine will be invaluable to the growth of fundamental science for the Gene Therapy Institute. For example, emerging molecular genetic analyses are likely to identify new gene therapy targets that can take advantage of the Gene Therapy Institute platform to perform fundamental experiments towards the development of new gene therapy interventions, facilitating 'bench to bedside' translational science as a collaborative effort between the Institute for Genomic Medicine and the Gene Therapy Institute. In addition, the resources and infrastructure of the two institutes will be additive and non-overlapping.

The Ohio State University's Center for Clinical and Translational Science. The Ohio State University Center for Clinical and Translational Science is a collaboration among The Ohio State University, The Ohio State University Wexner Medical Center and Nationwide Children's Hospital dedicated to turning the scientific discoveries of today into life-changing disease prevention strategies and the health diagnostics and treatments of tomorrow. The Center for Clinical and Translational Science provides financial, organizational, and educational support to biomedical researchers, as well as opportunities for community members to participate in credible and valuable research. Funded by a multi-year Clinical and Translational Science Award from the NIH, the Center for Clinical and Translational Science leverages expertise from every college across the University, including scientists and clinicians from the 7 Health Science Colleges, the College of Engineering, the Wexner Medical Center, Nationwide Children's Hospital, community health and education agencies, business partnerships, and regional institutional network partnerships.

Critical opportunity. The Center for Clinical and Translational Science interacts with scientists from all disciplines and, as such, plays a critical role in facilitating collaboration and broad educational programs. The advancement of gene therapy will utilize the Center for Clinical and Translational Science platform to engage current and newly recruited faculty with interests in gene therapy. In addition, existing education platforms for faculty and trainees could incorporate gene therapy.

The James Cancer Hospital and Solove Research Institute. The James Cancer Hospital and Solove Research Institute is a transformational facility within The Ohio State University Comprehensive Cancer Center that integrates scientific research, education and innovative patient care. Research and education spaces are located on every inpatient floor, enhancing our bench-to-bedside approach to patient care and giving patients early access to new targeted treatments and tools. The Institute manages hundreds of active and pending cancer clinical

trials, many of which are initiated by The Ohio State University Comprehensive Cancer Center (the James Cancer Hospital) investigators. This genetic- and molecular-based research helps us understand each patient's unique cancer and develop and deliver the most effective targeted therapy that leads to better outcomes, fewer side effects and more hope. In addition to being the third largest cancer hospital in the nation with 14 operating rooms, 6 interventional radiology suites and 7 linear accelerators for radiation therapy, the Institute includes a dedicated early-phase clinical trials unit.

Critical opportunity. Gene therapy in cancer is an important area of emphasis for the Gene Therapy Institute and interaction with The Ohio State University Comprehensive Cancer Center (the James Cancer Hospital) will facilitate advancement of new therapies. The synergistic infrastructures of the Gene Therapy Institute and by The Ohio State University Comprehensive Cancer Center (the James Cancer Hospital) will provide researchers with opportunities to translate therapies in unique disorders.

Goals Unaddressed by Existing Units

The University gene therapy research community has several critical needs that the Gene Therapy Institute will fulfill. These broadly fall into the themes of a collaborative community, clinical infrastructure, business model development and an education curriculum. These needs have been defined by requests from University faculty and are contained within this proposal. To fully maximize the University-wide expertise in gene therapy, it is critical to provide an Institute structure and administrative support across the continuum of science from training to product development (**Figure 2**).

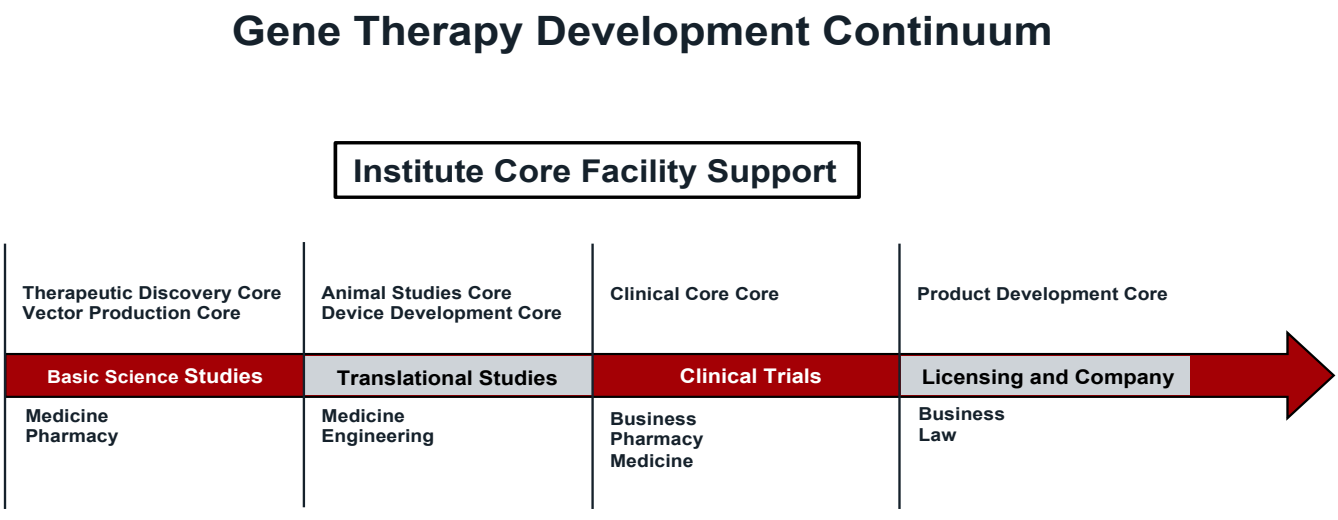


Figure 2. Continuum from gene therapy discovery to clinical product development demonstrating the critical input over time of Institute core support and the critical need for expertise from the various affiliated Colleges. The support and expertise are essential for effective and efficient progress across the related gene therapy programs.

III. Faculty

Criteria for Institute Membership

There are various categories of membership in The Ohio State University Gene Therapy Institute, including faculty, trainee, staff and external. Membership requirements are designed to be inclusive and based on a criterion of mutual benefit (i.e., the Institute and the faculty both advance their missions). Members must be involved in research, clinical activities or business activities related to gene therapy. Members are expected to engage in Institute activities, such as seminars, workshops, committees, education/training and outreach.

Faculty member. Faculty members of the Gene Therapy Institute are selected from faculty across the University Colleges. Institute faculty must have an expressed interest and/or expertise in the development of gene therapy and/or related fields, including the development of programs that advance the development and application of gene therapy. Faculty members must be Ohio State faculty (e.g., principal investigator, research scholar, research associate) responsible for research and/or education. Faculty members can be actively involved in basic, translational or clinical research related to gene therapy. Faculty members can be involved in the advancement of gene therapy programs through legal and/or business-related interest and/or expertise (e.g., operational excellence, intellectual property development, public-private partnership development, regulatory excellence, innovative business development).

Trainee member. Trainee members can include undergraduate, graduate or professional students at The Ohio State University with an interest in gene therapy research and/or programmatic development. Post-graduate (research, clinical [resident or post-resident fellows] or interns) trainees with interest in gene therapy are candidates for trainee membership.

Staff member. Staff involved in the support of a faculty member and their gene therapy program(s) are candidates for membership.

External members. Individuals actively involved in basic, translational or clinical research in gene therapy that are not primarily affiliated with The Ohio State University. Appointment to this category requires approval by Institute leadership (as defined below). While external members may participate in seminars and other center activities, they are not eligible to receive financial benefits.

Membership Review Procedures

To fulfil the mission of The Ohio State University Gene Therapy Institute, the Director, with support from the Gene Therapy Institute leadership, will review the participation of each member on an every 2-year review that will assess the following criteria:

- Serve as a mentor to gene therapy scientist or trainee, or
- Serve on an Institute committee, or
- Contribute to an Institute grant proposal as Principal Investigator, Co-Investigator, or contributing faculty, or
- Contribute to the Institute resource development efforts in business, intellectual property law, regulatory policy, operational efficiency, or
- Contribute to Institute curriculum, short-courses and/or workshops, or
- Serve as an Institute consultant to study design and/or analysis

After 2 years of inactivity, membership will be abolished if extenuating circumstances are not communicated and/or accepted. Upon loss of membership, the individual will not qualify for certain incentives of the Institute, such as seed grants, support for proposal development, access to core facilities and consulting services. Membership may be reinstated after a minimum of 1-year demonstrated activity to the criteria above and formal petition to the Director.

Institute Inaugural Faculty

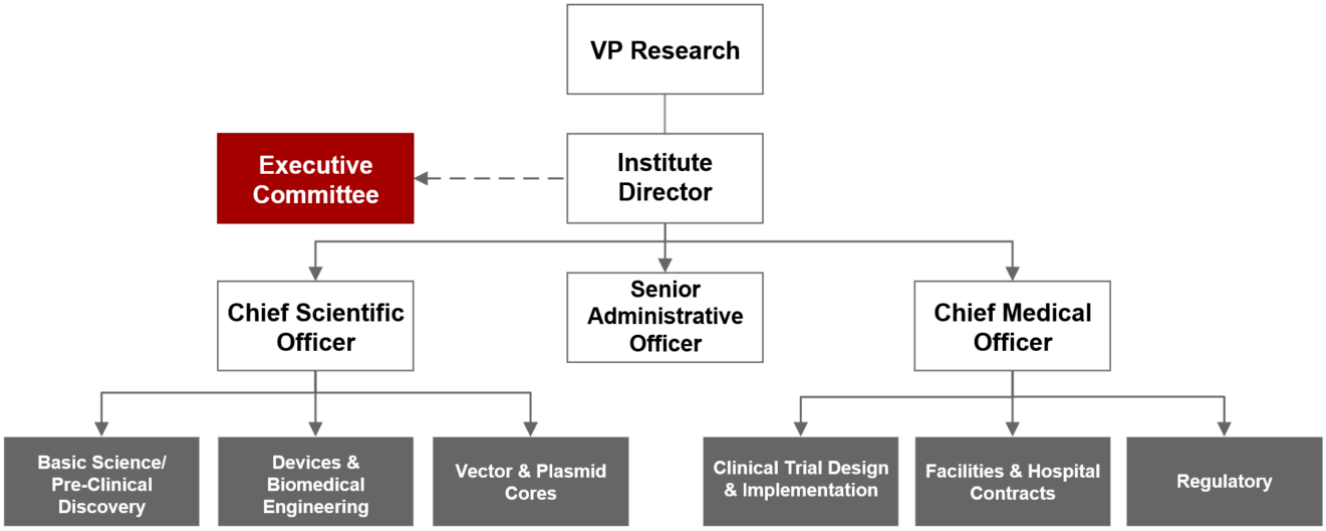
A complete listing of the Gene Therapy Institute faculty is provided in **Appendix A**. These faculty have interest and expertise (defined by the criteria above) in gene therapy programmatic development. Accompanying letters of support from Deans, Chairs and Directors of each of the colleges, departments and institutes are provided in **Appendix B**.

IV. Administration

Institute Administrative Structure

The leadership of the Gene Therapy Institute will report through the Office of Research within the Enterprise for Research, Innovation and Knowledge with a direct reporting line to the Vice President of Research. The organizational structure is designed to empower research and translation to impact the lives of patients. The Institute's organizational structure includes a Director who reports to the Office of Research, three Officers (Chief Scientific Officer, Chief

Medical Officer, and Senior Administrative Officer). The leadership will be expected to provide reports to key university leaders and leadership committees including the University Senate, Council of Deans, etc.



Institute Leadership

Institute Director. Russell Lonser, M.D., (Professor and Chair, Department of Neurological Surgery, The Ohio State University), will serve as Director of the Gene Therapy Institute. The Director works closely with Chief Medical Officer, Senior Administrative Officer, Business Manager and Chief Scientific Officer to advance the basic, translational and clinical research and education missions of the Institute.

Responsibilities of the Director, include:

- Oversee all aspects of Institute operation to strengthening the position of Ohio State as a leader in gene therapy.
- Develop and implement strategy and tactics, in conjunction with Institute faculty, to achieve Institute goals.
- Foster a culture of excellence based on integrity, sound fiscal policy and accountability.
- Represent the Institute within the university community and to the greater scientific community.
- Implement policy decisions made in consultation with Institute leadership and the executive and associate directors, and, where possible, after advisement from the executive advisory committee.
- Be a model of leadership in research and education in gene therapy internally and externally.

Institute Officers. The Ohio State University Gene Therapy Institute will have the following three senior leadership roles (in addition to Director), including Chief Scientific Officer, Chief Medical Officer, and Senior Administrative Officer.

Chief Scientific Officer. Krystof Bankiewicz, M.D., Ph.D., (Professor, Department of Neurological Surgery, The Ohio State University) will serve as Chief Scientific Officer for the Gene Therapy Institute. The Chief Scientific Officer works closely with Chief Medical Officer, Senior Administrative Officer and Director to advance the basic, translational and clinical research and education missions of the Institute.

Responsibilities of the Chief Scientific Officer include:

- Develop and implement strategies to position the Institute as the global leader in gene therapy research.
- Lead the recruitment and retention of diverse world-class gene therapy researchers.
- Build research platforms to facilitate gene therapy research and collaborations.
- Implement cores to produce plasmid and viral vectors.
- Lead cross Institute collaborations and programs that advance gene therapy research and programs.
- Manage Institute research staff.

Chief Medical Officer. Richard Gumina, M.D., Ph.D., (Associate Professor, Department of Internal Medicine Division of Cardiovascular Medicine) will serve as Chief Medical Officer for the Gene Therapy Institute. The Chief Medical Officer works closely with the Chief Scientific Officer, Senior Administrative Officer and Director to advance the implementation of clinical trials and the education missions of the Institute.

Responsibilities of the Chief Medical Officer include:

- Develop and implement strategies to position the Institute of Gene as the global leader in clinical trial research.
- Develop the structure and manage the clinical trials unit of the Gene Therapy Institute.
- Build research platforms to facilitate clinical gene therapy research and collaborations.
- Oversee regulatory compliance for all clinical investigations.
- Oversee all related hospital facilities and contracts.
- Manage Institute clinical research staff.
- Ensure highest quality for clinical gene therapy clinical operations.

Senior Administrative Officer. TBD will serve as the Senior Administrative Officer for the Gene Therapy Institute. The Senior Administrative Officer works closely with the Chief Scientific Officer, Chief Medical Officer, Business Manager, and Director to oversee and advance the implementation of the academic and business aspects of the Institute mission.

Responsibilities of the Senior Administrative Officer include:

- Develop and implement strategies to position the Institute as the global leader in gene therapy.
- Manage overall operations of the Institute.
- Drive operational excellence across all facets the Institute program.
- Lead and manage outreach and partnerships with Industry.
- Develop talent recruitment and retention strategy with Director and other Institute leaders.
- Oversee and expand development opportunities to support with Institute.
- Analyze, create and negotiate sustainable operating and funds flow model for gene therapy facility through development, clinical margin, research contracts and outside partnerships.

Executive Committee. The composition of the Executive Committee will represent the interests of the Gene Therapy Institute faculty, Colleges and University. The initial composition of the Executive Committee will be determined by the inclusion of one faculty member from each of the colleges with representation in the Institute (i.e., Medicine, Law, Business, Engineering, Pharmacy, as defined by College Deans) and one senior college leader (defined by Vice President of Research) to form a committee with a total membership of six members. The Institute Director will make recommendations for Executive Committee membership. Executive Committee membership terms are two years and renewable up to a maximum of six years upon majority vote approval by the Executive Committee members.

The Executive Committee will work with the Director and Institute leadership to:

- Support the Institute in the development and implementation of strategic plans.
- Support clinical, scientific and education programs.
- Engage in outreach within the university and broader community.
- Assess and address annual reviews of the performance of the Institute.

Reporting Structure

The Ohio State University Gene Therapy Institute will report through the Office of Research at the Ohio State University. The Director will report to the Vice President for Research (Peter Mohler, Ph.D.) within the Office of Research. The Director will work with the Vice President for Research to formalize budgets and set strategies in alignment with the Office of Research strategic priorities.

Pattern of Administration

The pattern of administration is as described above.

V. Budget

Overarching Programmatic Support

Strategic direction from University leadership, including the President, Executive Vice President for Research, Innovation and Knowledge, and the Vice President of Research, was to foster and grow a university-wide program in gene therapy led constituent faculty to develop an Ohio State University Gene Therapy Institute. This Institute would provide the infrastructure and support to accelerate science, develop transformative treatments and create innovative partnerships/paradigms that positively impact patients and position the University as a leader in the gene therapy. To lead in this rapidly accelerating domain requires talent, vision and rapid investment. The depth and breadth of talent, educational programs, partnerships and technologies across the entire university necessitate this structure as a university institute to both accelerate and accentuate the impact of this work for research, educational and clinical impact.

Budget

Pending approval of the Institute, Dr. Lonser will work with stakeholder colleges, Wexner Medical Center and University to support faculty and staff recruitment, space, and others. The College of Medicine, Wexner Medical Center, and Office of Research will share the administrative support and ramp up costs until fully operational. Over the past three years, significant philanthropy has been created to support gene therapy efforts and this will continue both for capital as well as operational support. Please see **Appendix C** for additional details on the initial three-year operating budget.

Sources. The Office of Research has committed an initial three-year investment of $333,000 per year. The College of Medicine will allocate existing support in the amount of $200,000 per year for operating expenses from research start up packages to support the Chief Scientific Officer and Chief Medical Officer salary expenses.

Uses. The majority of initial expenses will be to support the administrative structure of the Institute during the initial ramp up period. **It is expected that ongoing clinical trials and prior institutional investments will fully fund the proposed budget by FY25.** Additionally, current research projects, clinical trials and contracts are expected to create a significant margin that will be used to support growth and expansion of the Institute.

Personnel. Institutional support is requested to initially fund the administrative structure of the institute and assumes 10% effort for the Director, 20% effort for the Chief Scientific and Medical Officers, and 100% effort for the Senior Administrative Officer. The listed budget includes salary and benefit expenses. It is anticipated that faculty recruitment expenses will be shared with faculty TIUs and stakeholder colleges.

Operating Funds. The Gene Therapy Institute is requesting $100,000 per fiscal year to host a three-day annual symposium that would promote the Institute both to external institutions and within Ohio State to encourage interdisciplinary efforts. An additional $100,000 per fiscal year is budgeted for travel and marketing expenses.

Seed Funds. In order to support the Institute's Education and Training mission, $100,000 of support is budgeted to provide investigator seed grants and trainee support.

Facilities, Equipment and Resources. The existing infrastructure at Ohio State can currently support the proposed mission of the Gene Therapy Institute. Within the first fiscal year of operation, the Institute Director and Officers will collaborate with the Executive Advisory Committee to develop a model for growth and subsequent infrastructure needs.

A proposal has been submitted to create a Gene and Cell Based Therapies Neighborhood within the Interdisciplinary Research Facility that would provide additional infrastructure to support faculty members within the Institute.

Sustainability and Fiscal Stewardship

Extramural Federal Funding. NIH investment for gene therapy research projects has doubled, growing from $6.2B across 15,238 funded projects in 2011 to $13.2B across 25,788 funded projects in 2020. Focusing on neurological disorders alone, the most growth in funding was observed, expanding from $438M across 1,367 projects in 2011 to $1.07B across 2,239 projects in 2020.[4]

The faculty members of the Ohio State University Gene Therapy Institute have extensive experience with the NIH, and given the interdisciplinary nature of the newly formed Institute, the extramural grant portfolio will increase. The Institute will have broad impact across the university environment and the increased extramural grant revenue will benefit each home department and college.

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[4] "NIH RePORTER." *National Institutes of Health*, U.S. Department of Health and Human Services, 2022, https://reporter.nih.gov/search.

The funding strategy will entail continued focus on the NIH as we increase the breadth and depth of gene therapy research faculty by both bringing current faculty together in a collaborative environment, coupled with targeted recruitment of faculty that will serve as leaders and force multipliers. Additionally, the Gene Therapy Institute will bring engineering and medicine together to approach the complex problems of gene therapy delivery. These new approaches will be of great interest to industry partners and will result in grants to the National Science Foundation (NSF) and other federal funding opportunities. For example, Cedars-Sinai Regenerative Medicine Institute was awarded a $2.5M grant from the Department of Defense for a gene therapy study relating to Lou Gehrig's disease.[5]

Foundation Funding. Given the broad impact of potential gene-based cures and technologies, the foundation funding opportunities are significant and vast. The American Society of Gene and Cell Therapy features at least 10 large foundations providing funding opportunities for gene-based research.[6]

Industry Partnerships. The Ohio State University Gene Therapy Institute is positioned to establish sources of revenue from external partners, while continuing to serve the academic and training missions of the faculty and University. Leaders of the Gene Therapy Institute have ongoing relationships with the following industry partners in gene therapy:

- Bayer Corporation (funding 2 gene therapy first-in-human clinical trials for regeneration in multisystem atrophy and Parkinson's disease at The Ohio State University Wexner Medical Center).
- Voyager Therapeutics (funding 2 gene therapy first-in-human clinical trials for symptomatic improvement in Parkinson's disease at The Ohio State University Wexner Medical Center).
- uniQure, Inc. (funding a gene therapy first-in-human clinical trials for reversal of Huntington's disease at The Ohio State University Wexner Medical Center).
- Medtronic, Inc. (funding preclinical robotic device development investigations for gene therapy delivery).

Vector Core. The vector core provides an important resource to the Gene Therapy Institute faculty members. Additionally, the core will be developed into fee for service entity for outside collaborators and industry partners. Research-grade vectors will be provided to researchers

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[5] Cedars-Sinai Medical Center, "2.5 Million Dollar Defense Department Grant Funds Gene Therapy Study for Lou Gehrig's Disease." Cedars-Sinai Medical Center, 1 Apr. 2014, https://www.cedars-sinai.org/newsroom/25-million-dollar-defense-department-grant-funds-gene-therapy-study-for-lou-gehrigs-disease/.

[6] ASGCT. "Funding Opportunities: ASGCT - American Society of Gene & Cell Therapy." *American Society of Gene and Cell Therapy*, 2022, https://asgct.org/research/new-investigator-center-of-educational-resources/funding-opportunities.

across the world interested in gene therapy research. The vector core will become an approved facility to generate clinical-grade gene therapy products that could be monetized to support Institute mission.

Training. Based on the unique clinical expertise at the Gene Therapy Institute, there will critical opportunities to host workshops that provide training in gene therapy delivery. These training courses will generate revenue through registration fees.

Clinical Trials. Nationally, gene therapy clinical trials are supported by a variety of sources including the National Institutes of Health, academic centers, hospitals and industry (**Figure 3**).[7] This model of trial support is critical for effective fiscal stewardship and efficient translation of basic science discoveries into transformative clinical therapies. The Ohio State University Gene Therapy Institute will continue to perform clinical trials in this manner to exploit opportunities to perform clinical trials in a fiscally sound and effective manner.

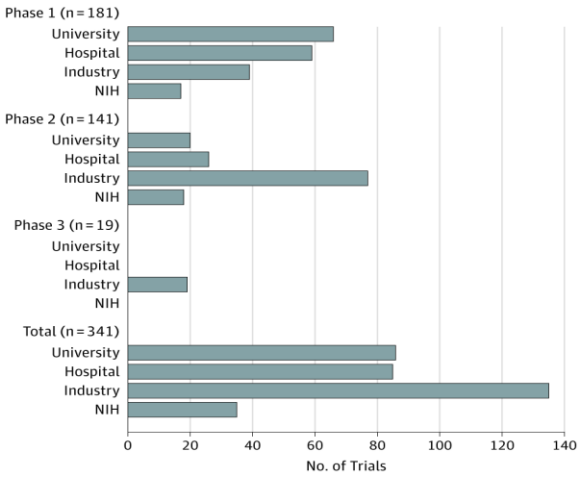

Figure 3. Breakdown of sponsorship for gene therapy trials in the United States.

Product Licensing and Company Development. The expertise and research findings derived from the Ohio State University Gene Therapy Institute faculty will drive critical discoveries and intellectual property in this area. This has been an excellent source of financial sustainability resulting from licensing fees, new company formation and other product development opportunities at academic institutions around the world.

[7] Z. Kassir, et al., Sponsorship and funding for gene therapy trials in the United States. ***JAMA*** 323:890-891, 2020.

VI. Evaluative Criteria and Benchmarks

Criteria

The criteria for evaluation of the success of The Ohio State University Gene Therapy Institute will include:

- Increased funding of Institute members.
- Increased number of collaborative grant applications.
- Increased number of publications in gene therapy scholarly journals with a priority in collaborative work (both total and high-impact publications).
- New intramural and extramural collaborations between Institute members, industry partners and/or investigators.
- Increased number of gene therapy related clinical trials (particularly first-in-human).
- Local, national and international honors and awards for Institute members.
- Improvements in student and postdoc training, measured by increased numbers and impact of publications and research presentations and successful job placement after graduation.
- Increased industry, non-profit, and government partnerships.
- Diverse and inclusive environment.
- Support of valuable gene therapy core facilities that can be shared by researchers (e.g., vector, therapeutic discovery/development, animal, device development, regulatory, clinical trial and product development; **Box 2**).

Benchmarks

Objective measurements for performance against the evaluation criteria, as follows:

- Extramural funding increase of more than 10% per annum.
- Increase and sustain Institute supported collaborative grant applications.
- A 20% increase each year in number of gene therapy publications.
- A 10% increase in membership each year, and demonstrated collaborations arising from Institute activities.
- A 20% Increase number of gene therapy related clinical trials.
- Recognition of gene therapy science excellence for numbers and prestige of awards bestowed.
- Academic excellence measured in publications (emphasis on high impact), student and post-doc career outcomes and growth of trainee led communities.
- New and sustained partnerships each year through formal agreements, contracts, or other engagements.

- Internal and external comparisons to measure underrepresented group representation in membership and leadership.
- Establishment and sustainability of gene therapy resources measured by increased use of resources and community feedback.

Evaluation Process

Per The Ohio State University Academic Center Guidelines, Faculty Rule 3335-3-36 Centers and Institutes, the Gene Therapy Institute leadership shall initiate a comprehensive self-study with guidance from at least two external reviews (identified by the Director and Executive Committee) aligned to the criteria for evaluation described above. The Institute Director will be responsible for obtaining performance metrics for awards proposed and awarded per annum, student performance and placement, and demonstrations of internal and external collaborations. Upon yearly review, the Director and Executive Committee will establish strategies to bolster areas of performance that do not meet or exceed benchmark criteria. Furthermore, as defined in 3335-3-6, the center will be reviewed two years after initial establishment and at 4-year intervals thereafter, as articulated in 3335-3-36.

VII. Supporting Material

Appendix A: Faculty Members

Faculty Name	Rank	Department	Other Title(s)
College of Medicine			
Krzysztof S. Bankiewicz, M.D., Ph.D.	Professor	Neurological Surgery	Gilbert and Katheryn Mitchell Endowed Chair Director, Brain Health and Performance Center
Brian Dalm, M.D.	Assistant Professor	Neurological Surgery	Director, Division of Neuromodulation
J. Bradley Elder, M.D., Ph.D.	Associate Professor	Neurological Surgery	Director, Division of Surgical Neuro-Oncology
Piotr Hadaczek, Ph.D.	Senior Research Scientist	Neurological Surgery	
Paco Herson, Ph.D.	Professor	Neurological Surgery	Associate Dean of Research Innovation Director, Innovative Partnerships and Analytics Director, Cerebrovascular and Neuroregeneration Institute
Russell R. Lonser, M.D.	Professor & Chair	Neurological Surgery	Dardinger Family Chair in Surgical Neuro-Oncology Co-director, Neurological Institute Faculty Affiliate, Chronic Brain Injury
Timothy Lucas, M.D., Ph.D.	Professor	Neurological Surgery	Executive Director, OSU-Battelle Neurotechnology Institute
Lluis Samaranch, Ph.D.	Assistant Professor	Neurological Surgery	
Kristy Townsend, Ph.D.	Assistant Professor	Neurological Surgery	

Victor Van Laar, Ph.D.	Assistant Professor	Neurological Surgery	
David Arnold, M.D.	Professor	Neurology	
Erica Dawson, Ph.D.	Associate Professor	Neurology	Director, Neuropsychology Director, Neurocognitive Wellness and Therapy program
Bakri Elsheikh, M.D.	Associate Professor	Neurology	
Yousef Hannawi, M.D.	Assistant Professor	Neurology	Director, Division of Stroke and Neurocritical Care
Stephen Kolb, M.D., Ph.D.	Associate Professor	Neurology	Director, ALS/MND Multidisciplinary Clinic & Translational Research Program
Sandra Kostyk, M. D	Professor	Neurology	Director, Center for Huntington's Disease
Ariane Park, M.D.	Assistant Professor	Neurology	Co-Director, Madden Center for Parkinson Disease & Other Movement Disorders
Kiran Rajneesh, M.D.	Assistant Professor	Neurology	Director, Division of Pain Medicine
Andrew Sass, M.D., Ph.D.	Assistant Professor	Neurology	
Douglas Scharre, M.D.	Professor	Neurology	Director, Division of Cognitive Neurology
Jan Schwab, M.D., Ph.D.	Professor	Neurology	William E. Hunt, M.D., and Charlotte M. Curtis Endowed Chair
Eric Bourekas, M.D., M.B.A.	Professor	Radiology	Chief, Division of Neuroradiology
Andrew Kalnin, M.D.	Associate Professor	Radiology	Director, Functional Magnetic Resonance Imaging
Michael Knopp, M.D.	Professor	Radiology	Novartis Chair of Imaging Research Director of the Wright Center of Innovation in Biomedical Imaging Faculty Affiliate, Chronic Brain Injury

Mona Natwa, M.D.	Professor	Radiology	Chief, Division of Nuclear Medicine
Luciano Prevedello, M.D., M.P.H.	Associate Professor	Radiology	Vice Chair, Informatics and Augmented Intelligence in Medical Imaging
Zarine Shah, M.D.	Associate Professor	Radiology	
Yin Ren, M.D., Ph.D.	Assistant Professor	Otolaryngology	
James Rocco, M.D., Ph.D.	Professor and Chair	Otolaryngology	Mary E. and John W. Alford Research Chair in Head & Neck Cancer
Kyle Van Koevering, M.D.	Assistant Professor	Otolaryngology	
Mohamed Abdel-Rahman, M.D., Ph.D.	Associate Professor	Ophthalmology & Visual Sciences	
Colleen Cebulla, M.D., Ph.D.	Professor	Ophthalmology & Visual Sciences	Torrence A. Makley Research Professorship Director, Lions Research Laboratory Director, Retina Research Laboratory
Nagaraj Kerur, Ph.D.	Associate Professor	Ophthalmology & Visual Sciences	
Thomas Mendel, M.D., Ph.D.	Assistant Professor	Ophthalmology & Visual Sciences	
Sayoko Moroi, M.D., Ph.D.	Professor & Chair	Ophthalmology & Visual Sciences	William H. Havener, M.D. Endowed Professor Chair and Director, Havener Eye Institute
Matthew Ohr, M.D.	Professor	Ophthalmology & Visual Sciences	
Michael Wells, M.D.	Associate Professor	Ophthalmology & Visual Sciences	
Kara Williams, M.S.	Assistant Professor	Ophthalmology & Visual Sciences	
Anthony Brown, Ph.D.	Professor	Neuroscience	

Jie Gao, Ph.D.	Assistant Professor	Neuroscience	
Jonathan Godbout, Ph.D.	Professor	Neuroscience	Faculty Director, Chronic Brain Injury Co-Director, Neuroscience Graduate Program Co-Director, NNDS T32 Training Program in Neuroimmunology
Hongjun Fu, Ph.D.	Assistant Professor	Neuroscience	
Dana McTigue, Ph.D.	Professor	Neuroscience	Associate Dean for Foundational Research Faculty Affiliate, Chronic Brain Injury
Karl Obrietan, Ph.D.	Professor	Neuroscience	Faculty Affiliate, Chronic Brain Injury
Giles Plant, Ph.D.	Professor	Neuroscience	
Philip Popovich, Ph.D.	Professor & Chair	Neuroscience	Executive Director, Belford Center for Spinal Cord Injury Director, Center for Brain and Spinal Cord Repair
James Comprehensive Cancer Center			
Lei Cao, Ph.D.	Professor		William C. and Joan E. Davis Cancer Research Professorship
College of Engineering			
Daniel Gallego-Perez, Ph.D.	Associate Professor	Biomedical Engineering	Associate Professor, General Surgery
Samir Ghadiali, Ph.D.	Professor & Chair	Biomedical Engineering	Professor, Internal Medicine
Natalia Higuita-Castro, Ph.D.	Assistant Professor	Biomedical Engineering	Assistant Professor, General Surgery
Moritz College of Law			
Efthimios Parasidis, J.D., M.B.E.	Professor		Professor, College of Public Health Faculty Affiliate, Center for Bioethics

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Rebecca Wolitz, J.D., M.Phil.	Assistant Professor		
Patricia J. Zettler, J.D.	Associate Professor		
College of Pharmacy			
Daniel Binzel, Ph.D.	Research Assistant Professor	Pharmaceutics & Pharmacology	
Ema Cocucci, M.D., Ph.D.	Assistant Professor	Pharmaceutics & Pharmacology	
Yizhou Dong, Ph.D.	Associate Professor	Pharmaceutics & Pharmacology	
Robert Lee, Ph.D.	Professor	Pharmaceutics & Pharmacology	
Jack Yalowich, Ph.D.	Professor	Pharmaceutics & Pharmacology	
Rebecca Lahrman, PharmD	Assistant Professor	Pharmacy Practice & Science	
Serena Zhao, PhD	Research Specialist		
Fisher College of Business			
Aravind Chandrasekaran	Professor		Professor of Operations Associate Dean for Graduate Programs & Executive Education

Appendix B: Letters of Support

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Appendix C: Initial Budget

	FY23	FY24	FY25
Sources			
Grants & Contracts (Core Directors)	$137,658	$ 141,099	$ 144,626
Office of Research	333,000	333,000	333,000
College of Medicine Start Up	200,000	200,000	200,000
Advancement Activity	200,000	200,000	200,000
Total Sources	**870,658**	**874,099**	**877,626**
Uses			
Personnel *(includes salary and benefits)*			
Institute Director	26,308	26,966	27,640
Chief Scientific Officer	52,616	53,931	55,279
Chief Medical Officer	52,616	53,931	55,279
Senior Administrative Officer	121,139	124,167	127,271
Core Director: Vector Production	137,658	141,099	144,626
Core Director: Animal Studies	137,658	141,099	144,626
Total Personnel	**527,993**	**541,193**	**554,723**
Operating Funds	200,000	200,000	200,000
Seed Funds	100,000	100,000	100,000
Total Uses	**827,993**	**841,193**	**854,723**
Net Margin	**$ 42,665**	**$ 32,906**	**$ 22,904**