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	Ref#	Project Name	Description	Primary Therapeutic Area	Secondary Therapeutic Area	Technology Type	Tag(s)	Lead PI Last Name	Lead PI First Name	PI Department	Stage of Development	Commercialization Focus
Recently Added	5949	Pittsburgh Performance Fatigability Index (PPFI) Diseases for Older Adults	Researchers have developed the Pittsburgh Performance Fatigability Index (PPFI), an objective and novel approach for tracking the performance fatigability in older adults (aged 60+). PPFI marks the first time that performance fatigability is derived from raw accelerometry measures. Using tri-axial raw accelerations from wrist-worn accelerometers, PPFI quantifies the percentage of area under the observed gait cadence. The system looks at time trajectory during a 400m walk to a hypothetical area that would be produced if the participant sustained maximal cadence throughout the entire walk.	Other		Other		Glynn	Nancy	GSPH-Epidemiology	Prototype	License
Recently Added	5916	Topical Formulation of B-Alanine toSuppress Mast Cell Activation in Rosacea and Other Diseases	Prior research at the University of Pittsburgh has demonstrated that b-alanine when administered by injection has beneficial effects in suppressing dermal mast cells. The development of a topical formulation has been hindered by the onset of transient itching that occurs following an application. To counteract this limitation, University of Pittsburgh researchers have created a formulation of b-alanine that includes the addition of an additive. With this additive, the formulation activates cool sensing neurons that suppress the sensation of itch induced by b-alanine. This additive is commonly found in creams but can release vapors that can result in eye irritation when applied on the face. However, Pitt researchers determined that 0.5% additive added to a 5% b-alanine topical cream formulation could reduce b-alanine induced itch, while not causing any eye irritation.	Dermatology	Immunology	Formulation	Consumer; Minimally Invasive	Kaplan	Daniel	Med-Dermatology	In vivo data	License;NewCo
Recently Added	5899	App Extends Rapid Relief From Treatment Resistant Depression Following Intravenous Ketamine	Pitt researchers utilized a computer-based intervention that is unique from other depression applications and software. The treatment was specifically designed to work synergistically with a rapid-acting psychiatric medication, such as ketamine to leverage rapid enhancements in neuroplasticity induced by the drug. The intervention then used adaptive learning to extend relief for patients. The software Pitt researchers created offers a simpler and more efficient system than most software programs targeting depression treatment. It administers an easy task that targets low-level classical conditioning processes to improve self-esteem, requiring minimal time and effort on the patient's part.	Neuroscience		Other	Digital Therapy; Combination Therapy; Synergistic Treatment; Neuroplasticity; Cognitive Training; Brain Training	Price	Rebecca	Med-Psychiatry	Prototype	License
Recently Added	5829	Novel combination therapy using IL-33 and Anti-Areg Antibodies Decreases Tumor Growth	Pitt researchers demonstrated that IL-33 induced strong CD8+ T cell antitumor immune responses characterized by robust clonal expansion and vigorous functional diversification. However, IL-33 also induced intense regulatory T cells accumulation in the tumor microenvironment (TME). These Treg cells expressed high levels of Areg protein. Lu's team further found that blockade of Areg protein further improved the antitumor efficacy of IL-33.	Oncology		Antibody,protein,peptide	Protein; Combination Therapy	Lu	Binfeng	Med-Immunology	In vivo data	License
Recently Added	5804	Xkr8 as a Novel Therapeutic Target for Cancer Immunochemotherapy	For the first time, Pitt researchers have shown that Xkr8 mRNA expression was significantly induced by chemotherapeutic agents in vitro and in vivo. With enhanced delivery of both types of therapeutics to tumors, this strategy has the advantage of selectively delivering Xkr8 siRNA to those tumor cells that are exposed to chemotherapy drugs. Codelivery of Xkr8 siRNA and 5-FU/oxaliplatin has led to significant improvement in tumor microenvironment and enhanced antitumor activity in colon and pancreatic cancer models. These results suggest Xkr8, in combination with chemotherapy, may be a novel strategy for treating additional cancers.	Oncology		Other	Drug Delivery	Li	Song	Pharm-Pharmaceutical Science	In vivo data	License
Recently Added	5763	Medium Chain Fatty Acids for the Treatment and Prevention of Acute Kidney Injury	University of Pittsburgh researchers tested this mechanism by feeding experimental animals dodecanedioc acid. They deduced that feeding them this acid blocks mitochondrial fatty acid oxidation and the associated oxidative stress and drives peroxisomal fatty acid oxidation. Pitt researchers administered this diet to the animals for one week before performing ischemia/reperfusion injury, simulating what is typically seen in a transplant, shock or trauma leading to AKI. Researchers found that the animals' kidneys showed dramatically less damage and had improved function. Animals were similarly protected from a second nephrotoxic form of AKI (as seen with chemotherapy agents). Administration of medium chain fatty acids has the potential to protect against kidney injury and the subsequent progression to CKD or end stage renal disease.	Nephrology	Metabolic Disease	Formulation	Medium Chain Fatty Acid; Therapeutic	Lucas	Sunder	Med-Pediatrics	In vivo data	License
Recently Added	5731	Dual Targeting Nanoparticles for Cancer Therapy	Pitt researchers have developed a nanocarrier that self assembles to form micelles, which are capable of codelivery of lipophilic/hydrophobic drugs and nucleic acids such as siRNA. The resulting co-loaded micelles are decorated with a mixture of CS and PEG-derivatized CS through charge interaction. Since tumor ECs express higher levels of CD44 than LSECs, Pitt researchers hypothesized that incorporation of appropriate amounts of PEG would minimize the interaction of the NPs with LSECs without significantly compromising the binding to tumor ECs or tumor cells. Preliminary studies have confirmed this theory by showing the optimization of the ratio of different components in the carrier led to significant increases in tumor accumulation with a concomitant decrease in liver uptake.	Oncology		Drug delivery	Polymer	Li	Song	Pharm-Pharmaceutical Science	In vivo data	License
Recently Added	5721	Promising Approach to Prevent Acute Lung Injury for Patients with Sickle Cell Disease	Recent clinical trial has shown a ~50% reduction in hospitalization of Sickle Cell Disease (SCD) patients receiving intravenous P-selectin Ab therapy, suggesting that therapies beyond P-selectin inhibition would be needed to prevent ACS in SCD patients. Researchers at the University of Pittsburgh have developed an approach to ameliorate the remaining (~50%) lung vaso-occlusion associated morbidity in SCD by inhibiting the Gasdermin-D (GSDMD) dependent signaling in leukocytes. The preliminary in vivo evidence using P-selectin-deficient SCD mice shows significant amelioration of lung vaso-occlusion and lung Injury following GSDMD-inhibition.	Respiratory		Antibody,protein,peptide	Antibody; Biologic	Sundd	Prithu	Med-Medicine	In vivo data	License
Recently Added	5703	Targeting LCN-2 as a Treatment for Eye Disorders	The invention is a method to target LCN-2 to treat age-related macular degeneration (AMD) by reducing or preventing the death of retinal pigmented epithelium (RPE) cells. The PIs have developed a neutralizing antibody to target LCN-2.	Ophthalmology	Immunology	Antibody,protein,peptide		Sinha	Debasish	Med-Ophthalmology		License
Recently Added	5522	Nampt Inhibitors Exhibit Excellent Potency and ADME Properties	Nampt is a rate-limiting enzyme in the Nicotinamide adenine dinucleotide (NAD+) salvage pathway that converts nicotinamide to nicotinamide mononucleotide in mammals. This in turn enables NAD+ biosynthesis. Pitt researcher's novel discovery showed the secreted form of the Nampt acts as a cytokine that stimulates NF-kb pathway and inflammation through activation of Toll-like Receptor 4 (TLR4).	Oncology	Immunology	Small molecule	Autoimmune	Chen	Beibei	Med-Medicine	In vivo data	License
Recently Added	5401	System and Method for Assessment of Stroke Patients and Personalized Rehabilitation	This invention includes a system and a method that enables objective assessment of stroke severity through measurement of the patient's ability to perform motions that are representative of normal daily activities. Stroke impairments are currently assessed by a clinician using the Fugl-Meyer Upper Extremity (FMUE) Assessment. While the FMUE scoring system is a good indicator of stroke severity, it relies completely on the clinician to complete and is not conducive to providing feedback to the patient or re-evaluation in at-home exercise settings. In addition, it has been pointed out that that the FMUE method has difficulty distinguishing the difference between improvement in motion vs strength and, therefore, compensatory strategies used by the patient to complete tasks can be difficult to detect. Beyond assessment, the method presented enables personalized rehabilitation in a connected at-home system. Based on the patient's results from the initial testing, the machine-learning based algorithm provides recommendations for rehabilitation exercises that are specifically tailored to the patient's unique capabilities.	Rehabilitation/Mobility				Sethi	Amit	SHRS-Occupational Therapy	Prototype	License
Recently Added	5281	Biodegradable Stimulator Leads for Pain	Pitt researchers are designing and will fabricate biodegradable leads using degradable metal as the conducting core and biodegradable polymer as the insulation. The stimulation capability and degradation profile will be optimized and tested in simulated body fluid first, followed by a pilot animal implantation and testing. These leads will provide medium-term pain relief for both acute perioperative neuropathic pain and chronic neuropathic pain states.	Other		Medical Device		Emerick	Trent	Med-Anesthesiology and Perioperative Medicine	Design	License;NewCo
Recently Added	5177	Novel Thiadiazines as Heat Shock Protein Modulators and Anti-Huntington Disease Therapeutic Agents	Using iterative alkylations, saponifications, and coupling reactions, Dr. Wipf and his team were able to synthesize novel thiadiazines in which the core heterocycle could be selectively functionalized. Previous analogs of these thiadiazines have been shown to act as agonists of Hsp70 and help to reduce alpha-synuclein aggregation associated with neurodegenerative diseases. This unique method of preparing and functionalizing synthetic thiadiazines has the potential to lead to potent and effective drugs to combat Huntington disease and other devastating neurodegenerative disorders.	Central Nervous System	Neuroscience	Small molecule		Wipf	Peter	Chemistry	In vitro data	License
Recently Added	4989	T Cell Receptors Targeting Mutations in RNA Splicing Factors	Pitt researchers isolated genes encoding two unique T cell receptors (TCRs) capable of recognizing peptide epitopes from a mutated RNA splicing factor commonly found in uveal melanoma, chronic lymphocytic leukemia, myelodysplastic syndromes, and breast cancer. When these genes were introduced into a donor T cell, mutation reactivity was conferred without eliciting reactivity against the non-mutated form of the protein. Cancer patients with similar mutations stand to benefit greatly from personalized immunotherapy focused on genetically engineering human T cells with TCRs targeted to this mutated RNA splicing factor.	Oncology	Immunology	Cell therapy	Biologic; Immuno-Oncology	Kammula	Udai	Med-Surgery	In vitro data	License
Recently Added	4988	T Cell Receptors Targeting Defective DNA Repair Proteins	Pitt researchers have isolated genes encoding two unique T cell receptors (TCRs) capable of recognizing peptide epitopes from mutated proteins found in many cancers. When introduced into a donor T cell, these genes conferred their respective mutation reactivity without conferring reactivity against nonmutated forms of the proteins. Cancer patients with similar mutations in their DNA repair proteins may benefit from genetic engineering of human T cells to express the unique TCRs, making it an attractive new targeted immunotherapy option.	Oncology	Immunology	Cell therapy	Biologic; Immuno-Oncology	Kammula	Udai	Med-Surgery	In vitro data	License
Recently Added	4564	Abdominal Aortic Aneurysm Prognosis Classifier	Abdominal aortic aneurysm (AAA) is the 13th leading cause of death in westernized countries with a mortality rate exceeding 85 percent. The prevalence of AAA is 8% for men over 65 years old in the United States and studies show it becoming more prevalent in women. Although several software packages have attempted to create a clinical finite element tool to predict rupture risk of AAA, these efforts have not gained significant traction in the clinical space, University of Pittsburgh researchers have found a solution: a methodology that relies on recent advancements in machine learning, which have yielded positive results in diagnosis and prognosis of diseases in patients. In this process, an aneurysm prognosis classifier (APC) will be trained on small aneurysms, which will create a robust risk score for clinicians to make decisions on costly surgical interventions. The data set will work with track changes to determine the shape of the aneurysm and biomechanical indices from various scans for a single patient at different time points, which will aid in determining the evolution of the aneurysm over time. A clinician would be able to decide on surgical intervention risk based on a variety of factors, rather than relying on the maximum diameter criterion. This methodology also creates a risk score that can save overall costs in patient management, as it's been reported that early endovascular repair reduces overall costs of AAA intervention when compared to direct costs associated with AAA monitoring over a 4-year period.	Cardiovascular		Clinical development tool	Artificial intelligence (AI); Computational biology; Machine learning	Chung	Timothy	Bioengineering	Initial prototype development, which entailed a first stage of training and internal validation; external validation study in progress	License
Recently Added	3579	Ventriculoamniotic Shunt for Fetal Aqueductal Stenosis (VASFAS)	This novel ventriculo-amniotic shunt represents a treatment breakthrough. Designed to meet specific performance requirements for in-utero drainage of CSF, VASFAS has a low profile to allow for percutaneous, ultrasound-guided placement, is atraumatic to brain and intrauterine tissues, and has systems to prevent dislodgement and clogging. VASFAS also features a one-way valve to prevent inflow of amniotic fluid into the cerebral ventricles. Combined with technology to detect and select appropriate AS cases, VASFAS has the potential to allow for full-term progression of pregnancies and preservation of neurological function during prenatal development.	Pediatrics/Neonatology		Medical Device		Chun	Young	Industrial Engineering	In vivo data	License

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5857	Using STAT5 Inhibitors for the Treatment of Cardiovascular Calcification	University of Pittsburgh researchers have discovered that early in osteogenic differentiation of valve interstitial cells and vascular smooth muscle cells, telomerase reverse transcriptase (TERT) interacts with Signal Transducer and Activator of Transcription 5A/B (STAT5) to bind to the promoter of RUNX2. RUNX2 is the master transcription factor required for osteogenesis. Disrupting this interaction is a targeted approach to stall or prevent osteogenic reprogramming of cardiovascular cells and cardiovascular calcification.	Cardiovascular		Small molecule		St Hilaire	Cynthia	Med-Medicine	In vitro data	License
5734	Blood test to detect cerebral aneurysm formation and progression	Pitt's proposed diagnostic test is a protein-based cytokine panel that uses whole blood. The test works by leveraging inflammatory cytokine profiles, which we have developed for the mouse model and retrospective human samples. Cerebral aneurysms are focal dilations of cerebral arteries that are present in 2-5% of the general population. Almost 95% of these vascular lesions are sporadic while the remaining 5% can be attributed to familial, infectious or traumatic causes. More than 90% of patients harboring aneurysms are undiagnosed until the aneurysm ruptures or is discovered incidentally. These lesions can rupture resulting in devastating subarachnoid hemorrhage leading to near 50% mortality and morbidity. This technology will allow for early detection and treatment.	Neuroscience	Cardiovascular	Other diagnostic	Cerebral aneurysm; Blood Test; Diagnostic; Cytokine Panel; Neurology	Friedlander	Robert	Med-Neurological Surgery	In vivo animal and human data	License
5717	PSMA with Multiple Single-Chain and Domain Binders Treats Prostate Cancer	This innovation regarding PSMA antibodies has several unique features. Researchers used the small format of the VH-antibody domains binders, which provides the opportunity for developing ADCs with better tumor penetration compared to the full IgG, or Fab, or scFv formatted ADCs. The antibodies developed in the invention are derived from fully human phage display libraries, which provides for low risk of inducing immune response. The selection of binders competing with the antibodies J591 and VH-PSMA from TNB585 ensured targeting distinct epitopes on the PSMA surface. The affinities of the selected binders span two orders of magnitude including the affinities of the reference binders. This provided for a tuning capability of the developed antibody-based pharmaceuticals. The developed PSMA antibodies can be used for targeting PSMA expressing cells with high selectivity using either ADC, or ADCC, or CAR T cells.	Oncology		Antibody,protein,peptide	Antibody; Biologic; Immuno-oncology	Dimitrov	Dimiter	Med-Medicine	In vitro data	License
5716	Enzyme Inhibitor Promotes Anticancer Immune Response	Researchers at the University of Pittsburgh have identified a novel enzyme inhibitor that promotes antitumor immune response. This inhibitor blocks activity of aldehyde dehydrogenase-1A enzymes. These enzymes synthesize retinoic acid to regulate multiple cellular processes. When applied to immune cells, this enzyme inhibitor enhanced dendritic proliferation and drove T cell differentiation away from immunosuppressive regulatory T cells and towards cytotoxic T cells. Treatment of cancer cells induced immunogenic cell death. When applied in vivo along with a tumor vaccine this enzyme inhibitor significantly increased tumor control. These successes make this novel enzyme inhibitor a promising candidate for developing immunotherapies that act on aldehyde dehydrogenase-1A to treat cancers that have yet to be successfully treated with immunotherapy.	Oncology	Infectious Disease	Small molecule	Immunotherapy; Immune Activator; Vaccine	Buckanovich	Ronald	Med-Medicine	In vivo data	License
5700	Human Antibodies Identified as Brain Cancer Immunotherapy Targets	Researchers at the University of Pittsburgh have identified eight human antibody heavy chain variable domains that have strong immunotherapeutic potential for treating medulloblastoma. These antibodies act against the oncogenic protein, protogenin. The eight identified antibody heavy chain variable domains act against different immunoglobulin and fibronectin domains of protogenin to block protogenin affected growth of medulloblastomas. These antibodies have been applied in vitro and have been shown to reduce the growth of group 3 medulloblastomas. The strong activity of these antibodies against protogenin makes them a promising immunotherapy target for treating medulloblastoma.	Oncology		Antibody,protein,peptide	Antibody; Pediatrics; Rare Disease	Dimitrov	Dimiter	Med-Medicine	In vitro data	License
5658	Microfluidic System Rapidly Isolates Extracellular Vesicles from Biological Fluids	Researchers at the University of Pittsburgh have developed a device and accompanying control software that isolates extracellular vesicles at specified size fractions from biological fluids within seconds. This hierarchically structured microfluidic fractionation device simultaneously isolates the entire size range of extracellular vesicles, including exosomes, microvesicles, apoptotic bodies, and large oncosomes, from any biological fluid. The device allows the user to control the size-specificity so that it can also be used to isolate large cells, such as oocytes and megakaryocytes, that are filtered out of biological fluids at the beginning of most analytical methods. The entire system is automated, has a high isolation throughput, and runs within seconds.	Other		Other	Extracellular Vesicles; Label-free Isolation; Cell Fractionation	Hajipouran Benam	Kambez	Med-Medicine	Prototype	License
5603	Inhibition of Fgr Reduces Radiation-Induced Profibrotic Markers	Using RNA-sequence analysis with a pure population of sorted radiation-induced senescent cells, University of Pittsburgh researchers found that Fgr mRNA is upregulated by more than 70-fold exclusively in senescent cells. Previous research does not implicate Fgr in RIS-induced lung fibrosis. Pharmacologic inhibition of Fgr in senescent cells reduced profibrotic gene expression in targeted cells, heralding the potential to prevent fibrosis from developing in individuals who have sustained radiation damage.	Respiratory		Small molecule		Mukherjee	Amitava	Med-Radiation Oncology	In vitro data	License
5579	Novel Approach to Discovering Repurposed Drugs and Compounds for Treatment against SARS-CoV-2 Infection	Rather than targeting viral proteins, Pitt researchers aim to exploit the natural antiviral programs that arm host cells and used a comprehensive, mechanism-unbiased, and highly integrated systems-level approach. A set of 38 priority candidate compounds targeting the host system, including repurposable and computational drugs, were identified using computational modeling. Fifteen compounds have potential antiviral actions, while 23 have possible anti-hyperinflammatory capabilities. Fourteen have been selected for in vitro assays with different cell lines. Several of these compounds inhibited SARS-CoV-2 infection in a dose-dependent manner, with two showing particular efficacy. These findings expand the repertoire of drugs and compounds that can be repurposed or developed for treating COVID-19 either independently or in combination with each other.	Infectious Disease		Small molecule		Schurdak	Mark	Med-Computational and Systems Biology	In vitro data	License
5572	MyBP - A digital tool for individuals with hypertension	MyBP is an automated program that assists patients in routine and systematic HBPM. It is designed to be flexible to a person's own weekly schedule and provide timely feedback and data tracking to facilitate understanding and continued engagement. Testing and research conducted by the University of Pittsburgh inventors using MyBP has shown high patient engagement, high value ratings by patients, improved confidence in HBPM, and intentions to improve BP-lowering, healthy behaviors (eg, diet and exercise). In patients with uncontrolled hypertension, use of MyBP appears to lower blood pressure.	Cardiovascular		Other	Digital therapeutic	Muldoon	Matthew	Med-Medicine	Proof of concept demonstrated with patients.	License;NewCo
5563	New Methodology Increases NAMPT Activity for Treating Several Diseases including Autoimmune Conditions	The methods and materials for increasing NAMPT activity can be used to increase NAD+ levels to slow cognitive decline, vision loss, and/or hearing loss as well as to prolong lifespan. Pitt researchers created a critical document that outlines compounds having the ability to increase NAMPT activity within cells and/or within a mammal, and also methods for treating mammals having a condition responsive to an increase of NAMPT activity.	Neuroscience	Oncology	Small molecule		Chen	Beibei	Med-Medicine	In vitro data	License
5559	Small Molecule Allosteric Modulators of Class B GPCRs inthe PTHR and Method to Identify Them	The allosteric drugs disclosed here were designed to specifically modulate selected interactions with the PTHR upon altering its structural mechanics. This "allo-targeting" approach is being hailed in drug discovery and development for its use in designing efficacious therapies. A structure-guided computational approach was used to identify allosteric druggable sites in PTHR and to predict compounds that would bind to this site, and Pitt researchers discovered four small-molecule compounds that act as negative allosteric modulators of PTHR signaling. Further computational protocols indicated that the experimentally-identified small molecule drug candidates were predicted to bind to this site, lending more evidence to the use of this computational approach to discovering druggable sites in other class B GPCRs.	Endocrinology	Musculoskeletal	Small molecule	Bioinformatics; Small molecule	Sutkeviciute	Ieva	Med-Pharmacology and Chemical Biology	In vivo data	License
5557	A New Peptide to Treat Glioblastoma by Modifying the Tumor Microenvironment to Enhance Antitumor Immune Response	Pitt researchers have discovered three novel immunomodulators of the TME that form protein binding complexes capable of controlling tumor-associated macrophages, which are the major components of the glioblastoma TME. This discovery enabled the design of a new peptide that interacts with the recently discovered immunomodulators to modify the TME to enhance antitumor immune response. Animal experiments revealed that this treatment produced an increase of T cell infiltration and expression of immune checkpoint inhibitors, suggesting the potential of the newly developed peptide to overcome GBM resistance to immunotherapy and synergize with immune checkpoint blockade therapy by combining with other FDA-approved drugs, such as nivolumab or pembrolizumab. This new peptide could give rise to a novel class of drug candidates, including small molecule compounds and therapeutic antibodies, for treating glioblastoma and other types of cancer.	Oncology		Antibody,protein,peptide		Hu	Baoli	Med-Neurological Surgery	In vivo data	License
5544	Antioxidant Therapy for Treating Propionic and Methylmalonic Acidemia	Researchers at the University of Pittsburgh have developed a new, non-invasive treatment for propionic and methylmalonic acidemia. Propionic acidemia is caused by a lack of the enzyme, propionyl-CoA carboxylase, which is key in metabolizing certain components of proteins and fatty-acid chains. Without this enzyme, reactive oxygen levels increase in cell mitochondria. This therapy is composed of mitochondria-targeted antioxidants that reduce levels of reactive oxygen in cells, thereby limiting the oxidation damage resulting from propionic and methylmalonic acidemia. By reducing reactive oxygen levels in cells, this therapy can mitigate physiological damage caused by these diseases.	Rare Diseases		Small molecule	Mitochondrial Targeted Antioxidants; Fatty Acid Oxidation; Oxidative Phosphorylation; Respiratory Chain; Electron Transport Chain	Vockley	Gerard	Med-Pediatrics	In vivo data	License
5535	Tissue Customized Platelet-Rich Plasma for Optimized Skeletal Muscle, Cartilage, and Bone Healing	In order to make PRP therapy as effective and beneficial as possible, a Pitt researcher has developed a novel approach to customizing PRP to be tissue-specific by eliminating negative/deleterious factors for specific tissue. This tissue-customized PRP will have targeted factors eliminated by neutralizing antibodies that are conjugated using magnetic beads. This technique can easily be added to current PRP isolation protocols, leading to the development of novel approaches to optimize the use of PRP through customization and minimizing potential deleterious effects on the targeted tissue.	Orthopedics		Cell therapy	Biologic	Li	Hongshuai	Med-Orthopedic Surgery	In vitro data	License
5523	300 Novel CB1 Antagonists Hold Potential for new Treatments	Cannabinoid receptor type 1 (CB1) is a G protein-coupled cannabinoid receptor encoded by the CNR1 gene. Through a detailed process involving the synthetization and testing of hundreds of antagonists, University of Pittsburgh researchers have now completed testing of over 300 novel CB1 antagonists that are structurally distinct from all prior classes, which indicates this discovery holds vast potential for additional treatments and uses within the complex physiologic system.	Metabolic Disease		Small molecule	Small molecule	Chen	Beibei	Med-Medicine	In vitro data	License
5521	Cytosolic Protein Quality Control Small Molecule Therapeutics	Pitt researchers developed a high-throughput optical screen for such modulators and conducted a pilot screen that successfully identified several compounds of interest. There is a wealth of interest in small molecule PQC modulators of cytosolic proteins that may serve as therapeutic drug targets for diseases characterized by cytosolic protein instability.	Rare Diseases		Small molecule	Rare disease	Palladino	Michael	Med-Pharmacology and Chemical Biology	In vitro data	License
5520	Therapeutic Targets for TPI Deficiency	In order to identify novel factors that modulate mutant TPI turnover, University of Pittsburgh researchers performed a genome-wide RNAi screen targeting known and predicted quality control proteins. Of more than 400 proteins screened, 25 regulators of TPI were identified, ten of which were novel and previously undescribed. These ten proteins are also conserved in mice and humans, making them more likely to be effective drug targets than other more promiscuous proteins. The proteins identified by this study may reveal novel pathways and drug targets to block degradation of functional, cytosolic proteins, and represent important therapeutic targets for drug development for TPI deficiency and other devastating diseases.	Rare Diseases		Other	Rare disease	Palladino	Michael	Med-Pharmacology and Chemical Biology	In vivo data	License
5496	Driving Oxidative Metabolism in Therapeutic T Cells through Overexpression of AMPK	Cultured T cells that are capable of performing oxidative metabolism are more fit for the metabolic conditions they will encounter in vivo, and promoting oxidative metabolism has been shown to increase their in vivo persistence. Oxidative metabolism is also inversely correlated with how differentiated a cell becomes, and a major goal has been to make effector cells both less differentiated and more oxidative before transfer into recipients.Increasing the cellular energy sensor AMP-activated protein kinase (AMPK) is expected to increase the oxidative metabolism of a T cell, leading to an increase in both its stability and suppressive capabilities. By overexpressing the regulatory component of the AMPK heterotrimeric complex via lentiviral transduction of primary human T cells, Pitt researchers propose to modulate AMPK activity and thereby improve the efficacy of multiple therapeutic interventions dependent upon T cells.	Oncology		Cell therapy		Byersdorfer	Craig	Med-Pediatrics	In vitro data	License

5491	Odansetron for Treatment of Acute Kidney Injury	Using electronic medical records (EMR) from more than twenty thousand AKI patients in ICU stays, researchers identified a novel indication for odansetron, an antiemetic drugused to prevent nausea caused by chemotherapy, in preventing deathof patients with AKI. The molecular mechanism of odansetron suggests that it can down-regulate AKI-related genes. Odansetron's beneficial effects on recovery from acute injury have never been reported before.	Critical Care		Small molecule		Wang	LiRong	Pharm-Pharmaceutical Science	Retrospective clinical data	License
5450	RNF167 and CASTOR1 as Novel mTOR Targets	Low CASTOR1 expression is a poor prognosis marker for ten types of cancer, while high expression of the RNF167, a ligase that targets CASTOR1 for degradation, has been identified as a poor prognosis marker for five types of cancer. Researchers also demonstrated that AKT and RNF167-mediated CASTOR1 degradation activates mTORC1 and promotes breast cancer progression. AKT-mediated phosphorylation of CASTOR1 significantly increases its binding to RNF167, revealing a novel mTORC1 regulating mechanism and potential new therapeutic targets for mTORC1-dysregulated diseases.	Oncology		Small molecule		Gao	Shou-Jiang	Med-Microbiology and Molecular Genetics	In vitro data	License
5448	Stimulation of Angiogenesis with miRNA as Protection Against Acute Kidney Injury	One of the hallmarks of AKI is damage to renal microvasculature, which alters endothelial function and contributes to hypoxic and inflammatory injury to the renal parenchyma. In AKI, some miRNAs appear to act pathogenically by promoting inflammation, apoptosis, and fibrosis, while others may confer protective benefits. Researchers have obtained preliminary data indicating that the absence of the miR-17~92 cluster makes cells are more susceptible to renal ischemia-reperfusion injury while miR-18a and miR-19b protects against renal IRI, providing evidence for a potential novel therapeutic approach for the treatment of acute kidney injury.	Nephrology		Other		Lucas	Sunder	Med-Pediatrics	In vivo data in mice using mimics	License
5404	Insertion Unique to SARS-CoV-2 Exhibits Superantigenic Characteristics	Using structure-based computational models, researchers have demonstrated that the SARS-CoV-2 spike harbors a sequence motif unique to SARS-CoV-2 and not present in other SARS coronaviruses, which is highly similar in both sequence and structure to bacterial superantigens. Further examination suggested that the SARS-CoV-2 spike may act as a superantigen that drives the development of MIS-C as well as the cytokine storm in adult COVID-19 patients. Potential development strategies include preparing a decoy peptide that can bind to the site in the viral structure and prevent it from binding to T cell receptors or preparing a monoclonal antibody specific to the viral superantigenic binding site and thus block the interaction with the T cell receptor.	Infectious Disease		Antibody,protein,peptide		Bahar	Ivet	Med-Computational and Systems Biology	Design; candidate antibodies identified	License
5390	Small Molecule Inhibitor Therapy to Prevent Aneurysm Formation, Growth, and Rupture	New research shows that small molecule inhibitors targeting the platelet-driven CXCL7-CXCR1/2 inflammatory pathway can be used to prevent cerebral aneurysm formation and rupture. This approach may be able to be used to develop a pharmacological treatment of unruptured and coiled aneurysms, enabling a superior healing response and avoiding the risks inherent in open surgery.	Neuroscience		Small molecule	Small molecule	Friedlander	Robert	Med-Neurological Surgery	In vivo data, including cytokine arrays and ELISA data from a hypertensive mouse model of intracranial aneurysm formation; electron microscopy of aneurysm samples, cytokine arrays of aneurysm samples, and blood samples from human patients with aneurysms, and in silico computational data for pathway discovery from the above cytokine arrays.	License
5372	HDAC Inhibitors as Anticancer Agents	University of Pittsburgh researchers have developed a series of chromane-based hydroxamic acids that have been demonstrated to be potent and selective HDAC inhibitors with potential use as novel anticancer and anti-neurodegenerative agents.	Central Nervous System		Small molecule		Wipf	Peter	Chemistry	In vitro data	License
5326	SARS-CoV-2 Recombinant Adenovector Vaccine	The spike (S) protein on the envelope of SARS-CoV-2 and other coronaviruses has been identified as a mediator of viral entry into a host cell, and it has been demonstrated that antibodies targeting the S protein can block the binding of these viruses to the cell receptor. Further, targeting the S1 subunit of the S protein generates a more efficacious neutralizing antibody response than targeting the full-length S protein in addition to reducing the potential risk of antibody-dependent enhancement observed with some vaccine candidates that targeted the entire S protein. Pitt researchers have constructed and evaluated a recombinant adenoviral vector encoding the transgene for the antigen SARS-CoV-2 S1 for COVID-19 which elicits a potent and specific IgG antibody response as early as two weeks after vaccination. They have also discovered that intranasal vaccine delivery generates significantly higher antigen-specific IgG levels and neutralizing activity compared to subcutaneous vaccine injection. This new adenovirus vaccine and its associated intranasal delivery mechanism display promising immunogenicity, making it an appealing candidate against this and other emerging coronavirus diseases.	Vaccines		Vaccine		Gambotto	Andrea	Med-Surgery	In vivo data	License
5317	A Novel Neoplastic Fusion Transcript Predicts Sensitivity to the MEK Inhibitor Trametinib in More Aggressive and Metastatic Breast Cancers	Researchers at University of Pittsburgh have discovered a non-traditional molecular event underlying molecular pathobiology of more aggressive and metastatic breast cancer. In this study, a large-scale analysis of breast cancer transcriptome revealed a tumor-specific fusion transcript that is preferentially overexpressed in luminal B and metastatic breast cancers and has been shown to increase aggressiveness of luminal breast cancer cells. This fusion also appears to activate a chain of signaling proteins that play a critical role for cancer cell to disseminate and colonize distant organs. To date, this fusion remains the most frequently expressed tumor-specific fusion transcript reported in luminal breast tumors. Importantly, breast cancer cells overexpressing this fusion transcript show markedly increased sensitivity to trametinib, the first FDA approved oral MEK inhibitor used for treating melanoma. This discovery suggests a new paradigm that non-traditional molecular events may be accountable for more aggressive and metastatic breast cancers and are a promising target for treating these deadly tumors.	Oncology		Antibody,protein,peptide		Wang	Xiaosong	Med-Pathology	In vitro data	License
5298	ImmunoPET Imaging of CD107a	A promising option to improve diagnostic imaging is immunoPET, which combines the high sensitivity and quantitative capabilities of positron emission tomography (PET) with the specificity and selectivity of monoclonal antibodies (mAb) against a given tumor cell surface marker. Targeting the cell surface marker CD107a, a marker of CD8+ T-cell degranulation and natural killer (NK) cell functional activity,with immunoPET probes can quantify the extent of T-cell mediated cytotoxic action, which directly correlates to immunotherapy.This technique serves as a novel diagnostic, a means of measuring immunotherapeutic response to treatment, and a non-invasive therapy. ImmunoPET imaging with CD107a mAbs represents a move away from a one-medicine-fits-all trial-and-error approach to treating cancer to offering the right treatment, for the right patient, at the right time, providing a more targeted, personalized, and efficient therapy.	Oncology		Molecular diagnostic		Edwards	Wilson	Med-Radiology	In vivo data	License
5290	Profilin1-actin interaction inhibitor as a Novel Anti-Angiogenic Compound	Profilin1-actin interaction is critical for actin-driven biological processes; specifically, angiogenesis, which drives ccRCC in addition to other pathologies including proliferative diabetic retinopathy, wet age-related macular degeneration, and other types of cancer. Targeting Profilin1 instead of VEGF is an alternative strategy to treating these diseases without developing the spontaneous or acquired resistance seen in anti-VEGF approaches. Proof-of-concept studies have demonstrated that inhibiting the Profilin1-actin interaction reduces proliferation and migration of RCC tumor cells and may also prove useful as a prognostic biomarker.	Platform Technology		Small molecule		Roy	Partha	Bioengineering	In vivo data; SAR studies of commercially available structural analogs underway	License
5259	Human Monoclonal Antibodies against SARS-CoV-2	Pitt researchers have developed neutralizing human mAbs that specifically target the SARS-CoV-2 RBD using large phage displayed antibody libraries for use in preventing and treating SARS-CoV-2. Two high-affinity binders neutralized the virus by competing with ACE2 for binding with the receptor. One other high-affinity binder did not compete significantly with ACE2, but could induce antibody-dependent cellular cytotoxicity (ADCC), killing infected cells. To our knowledge, these were the first human mAbs that can bind to the RBD and neutralize the virus.	Infectious Disease		Antibody,protein,peptide	Antibody	Dimitrov	Dimiter	Med-Medicine	In vitro data	License
5236	Substituted Indoles with Activity to Treat Acute Kidney Injury	Using a proliferation-based phenotypic assay in zebrafish, researchers have discovered a class of compounds which selectively inhibit HDAC and enhances recovery from acute kidney injury when given days after the initial injury.	Nephrology		Small molecule		Huryn	Donna	Pharm-Pharmaceutical Science	In vivo data in zebrafish	License
5224	RNA-and DNA-Based Assays for Predicting Paclitaxel Resistance in Triple Negative Breast Cancer	While the complexity of genomic rearrangements in this cancer has obscured the role that gene fusions play in the pathology of TNBC, researchers at the University of Pittsburgh identified 99 recurrent gene fusions, 57% of which are cryptic adjacent gene rearrangements (AGRs). The most frequently occurring AGRs were preferentially found in the more aggressive forms of breast cancers that lacked well-defined genetic targets; one was found exclusively in TNBC and TNBC tumors with this fusion gene exhibited aggressive histopathological features such as gross necrosis and high tumor grade. This fusion gene was also shown to endow resistance to paclitaxel treatment. RNA- and DNA-based assays for this gene fusion can be used to predict paclitaxel resistance in triple negative breast cancer and allow treatment providers to quickly pivot to alternative treatment options, sparing the patient from the unnecessary and unpleasant side effects of chemotherapy, in addition to serving as a target for novel therapeutics.	Oncology		Other diagnostic		Wang	Xiaosong	Med-Pathology	In vitro data	License
5214	Cationic Peptides for Treatment of Drug-Resistant Cancer and Infections	Peptide studies to date are incremental and tailored to a single best peptide. With this new technology, researchers designed a rational framework for engineering a class of cationic peptides with the property to overcome MDR bacteria-related infections. This means the invention can yield dozens of therapeutics, which display in vivo efficacy. The technology transcends the discovery of a particular drug and encompasses a class of compounds that is iterative.	Infectious Disease	Oncology	Antibody,protein,peptide	Biologic; Peptide	Deslouches	Berthony	GSPH-Environmental/Occupational Health	In vitro data	License
5099	Trabecular Meshwork Stem Cell Secretome for Treatment of Glaucoma	The secretome derived from trabecular meshwork stem cells (TMSCs) has been found to reduce IOP in two glaucoma mouse models of steroid-induced and inherited glaucoma. Treatment with secretomeby periocular injectionleadsto dramatic IOP reduction to a normal range for up to two months,as well as improved retina function similar to normal animals. Secretome reduced fibrosis in wounded TM cells, increased TM cells'wound healing capacity, and protected retinal ganglion cell from death. The safety evaluation did not indicate any side effects with secretome treatment.	Ophthalmology		Cell therapy		Du	Yiqin	Med-Ophthalmology	In vitro and in vivo data; xenograft experiments have been completed with very promising results	License
5088	New Gene Therapy Concept Shows Promise in Treating Age-Related Macular Degeneration	University of Pittsburgh researchers have developed several constitutively active forms of TFEB (Transcription factor, EB), a protein that in humans is encoded by the TFEB gene. This protein can translocate into the nucleus of retinal pigmented epithelium cells, and package them in AAV2 with a hVMD2 promoter. As a proof of concept, Pitt researchers used animal data that showed the active form of TFEB could rejuvenate the function in RPE cells and rescue the AMD-like phenotype.	Ophthalmology	Neuroscience	Gene therapy	Drug Discovery	Sinha	Debasish	Med-Ophthalmology	In vivo data	License

5084	Lung-Targeting Peptide to Deliver Diagnostic and Therapeutic Targets to the Lung	Building on previously synthesized cell-penetrating peptides, researchers at the University of Pittsburgh developed two new peptides that displayed up to five times greater transduction activity compared to its predecessors in vitro. Interestingly, these peptides show robust uptake in lung tissue and epithelial cells lining the alveoli far in excess of the heart—the expected target—and in excess of any uptake of the original peptide. Delivered via injection, these cell-penetrating peptides have previously demonstrated their capacity to act as vectors for delivery of genes, siRNA, anti-sense oligonucleotides, peptides, proteins, nanoparticles, viral particles, and radioisotopes. These novel synthetic peptides present a wealth of new opportunities for drug delivery to the lungs via peripheral injection, sidestepping the mechanical and immunological barriers that have thus far prevented efficient pulmonary drug administration.	Respiratory	Platform Technology	Drug delivery		Zahid	Maliha	Med-Developmental Biology	In vivo data using a chemical model of COPD	License
5075	Upregulation of NMDA Receptor Function by a GluN2A/ZnT1-directed Peptide	Researchers designed a cell and blood-brain-barrier permeable peptide termed TAT-N2AZ aimed at disrupting the ZnT1-GluN2A interaction. They observed that in the presence of the peptide, but not a control scramble, NMDA receptor activation was enhanced by decreasing the inhibitory actions of synaptically-released zinc. This is the first tool developed to enhance NMDA receptor function via a previous undescribed mechanism and may be useful in the treatment of disorders associated with NMDA receptor hypofunction,such as schizophrenia.	Neuroscience		Antibody,protein,peptide		Aizenman	Elias	Med-Neurobiology	In vitro data	License
5071	Oncolytic Vaccinia Virus Delivering Tethered IL-12 Enhances Antitumor Effects with Improved Safety	Pitt researchers constructed an oncolytic vaccinia virus that encodes membrane-tethered IL-12 and tested if it could turn a cold tumor into hot tumor while avoiding the systemic toxicity of IL-12. Virus-delivered IL-12 was shown to have greatly reduced toxicity, while retaining its potent capability of eliciting an antitumor immune response. The treatment facilitated the transformation of a cold tumor to a hot tumor and improved survival. Combined with PD-1 blockade, it induced potent antitumor effects in multiple tumor models. Impressive trials in mice suggest immediate translatability to clinical settings.	Oncology		Drug delivery	Immuno-Oncology	Bartlett	David	Med-Surgery	In vivo data	License
5061	Peripheral Nerve Agonists to Suppress Inflammation	Based on a discovery that neurons that promote painful sensations and also drive inflammation in the skin, researchers at the University of Pittsburgh determined that a specific subset of neurons that innervate the epidermis as well as the intestine are required to suppress the activation of inflammation-causing mast cells. This unique group of neurons express a protein which, when treated with its corresponding small-molecule agonist, suppresses cutaneous mast cell function. This discovery indicates that small-molecule agonists of this neuron type could be used to suppress mast cell activation without inducing global immune suppression, fulfilling an as-yet unmet therapeutic need.	Other		Small molecule		Kaplan	Daniel	Med-Dermatology	In vivo data including in mouse models of human rosacea, atopic dermatitis, general dermatitis, urticaria, and psoriasis	License
5010	A Novel Therapeutic Target and Clinical Marker for Pulmonary Arterial Hypertension	SCUBE1 gene has been shown to modulate pulmonary endothelial angiogenic potential, proliferation, and apoptosis. In PAH animal models and patients, SCUBE1 levels are decreased and negatively correlate with disease severity and progression, indicating its potential usefulness as a therapeutic target. By modulating pathogenic endothelial dysfunction and serving as a circulatory plasma marker for diagnosis of PAH, SCUBE1 could prove incredibly useful as a therapeutic target and for monitoring severity and progression of the disease.	Respiratory		Other		Chan	Stephen	Med-Medicine	In vivo data	License
4854	Novel Ticagrelor Coated Coronary Stent Using a Self-Assembled Monolayer Linker System	Researchers at the University of Pittsburgh have invented a novel vascular stent with inherent antiplatelet capabilities, which therefore requires no antiplatelet therapy after implantation. Ticagrelor, an anticoagulant frequently used in systemic antiplatelet therapy, is tethered to the surface of the stainless-steel stent using a chemical linkage with self-assembled monolayers, which prevents disaggregation of ticagrelor from the stent. This targeted approach mitigates bleeding risk associated with use of longer term systemic antiplatelet therapy and has the additional benefit of preventing blood-metal contact. This stent has been tested in rabbit implant studies and demonstrated 100% patency of the stent after 35 days with no systemic antiplatelet therapy.	Cardiovascular		Medical Device		Pacella	John	Med-Medicine	in vivo data	License
4771	Diagnostic for Preventing Necrotizing Enterocolitis (NEC)	Bacteria that escapesbinding by maternal antibodies such as IgA has been associated with later development of NEC. We have developed a bacterial array which allows for determination of the anti-bacterial repertoire--the number of different bacteriathat can be bound--of any given breast milk sample. The breadth of IgA specificity for these bacteria as determined by the array will indicate which milk samples are the most effective at preventing NEC, and will allow NICU doctors and donor milk banks to target milk samples to the most at-risk infants. The array is both customizable to customer needs and fast to run with a 24 hour turn-around time. The array can also be repurposed to combat other infant health risks, such as bacteremia and viremia.	Pediatrics/Neonatology		Other		Hand	Timothy	Med-Pediatrics	Device designed Proof of principle in place Post-hoc clinical trial pending	License;NewCo
4735	One-step Gene Therapy for Duchenne Muscular Dystrophy	The investigators designed a novel dual-cassette adeno-associated viral (AAV) vector therapy that combines gene therapies to recover dystrophin function, specifically targeting and reducing inflammatory responses associated with DMD. These vectors have been tested in mouse models of DMD. After a single dose, robust dystrophin expression was observed in both the cardiac and skeletal muscle of treated mice, in addition to reduced inflammation. The single-cassette with dystrophin gene replacement alone was not effective in reducing inflammation, indicating that this is the only DMD gene therapy that targets an inflammatory pathway in DMD that plays a key role in disease progression but while promoting dystrophin expression.	Rare Diseases		Gene therapy		Wang	Bing	Med-Medicine	Prototype, in vivo data collected	License
4731	Anti-Inflammatory Small Molecules for Treatment of Chronic Inflammatory Disorders	Researchers at the University of Pittsburgh have identified a new class of anti-inflammatory small molecule inhibitors that act on NF-κB to inhibit inflammatory immune responses. NF-κB are important regulators of cell responses, including the release of cytokine and other inflammatory mediators, and overactivation of NF-κB can lead to long-term, negative health impacts. By inhibiting the NF-κB pathway, this class of recently identified small molecules can reduce inflammation and prevent long-term health impacts and acute symptoms of inflammatory disorders.	Respiratory	Neuroscience	Small molecule	Neurological Diseases	Chen	Beibei	Med-Medicine	In vitro data	License
4729	Small Molecules Maintaining NMNAT2 Levels Prevent Degradation	Pitt researchers have undertaken a program to develop small molecules that augment NMNAT2 levels in the setting of neuronal injury. These approaches center on inhibiting Phr1 (MYCBP2), the E3 ubiquitin ligase that normally targets NMNAT2 for proteasomal degradation. During this process, small molecule inhibitors of Phr1/MYCBP2 will be identified, modified, refined and tested in relevant cell and animal models. While previous studies have sought to understand the molecular basis of Wallerian degeneration (WD), Pitt researchers have developed a first in class compound that holds the potential to prevent NMNAT2 degradation.	Neuroscience		Small molecule	Small molecule	Chen	Beibei	Med-Medicine	In vitro data	License
4712	CyteSolutionsLens: Drug-Eluting Contact Lens Technology	Developed at the McGowan Institute for Regenerative Medicine in conjunction with clinical experts from the University of Pittsburgh Medical Center's Eye & Ear Institute, the CyteSolutions Lens is a soft lens-based therapy and features use of a low-dose, sustained, locally releasing drug, providing convenient application with a familiar modality and long-term relief of symptoms. The CyteSolutions Lens releases a drug targeting a novel underlying pathway of dry eye inflammation not previously targeted in currently available therapies. Unlike competitors such as Restasis®, Cequa™, Xiidra®, and over the counter tear substitute eye drops, our therapy can be applied infrequently and overnight, with potential to provide days-long relief. Laboratory in-vitro tests have also shown ability of the CyteSolutions Lens to release the active ingredient for days, reducing frequency of treatment and creating longer lasting symptom relief.	Ophthalmology		Drug delivery		Nolfi	Alexis	Bioengineering	In vivo data	License;NewCo
4623	PDLIM2 Therapy for Cancer	PDLIM2 is a protein that acts as a tumor suppressor and whose expression is often repressed in various cancers. Repression of PDLIM2 is linked to cancer development, progression, metastasis, and therapy resistance, including complete resistance to anti-PD-1 therapy and epigenetic drugs. University researchers have developed several clinically feasible methods to restore PDLIM2 expression and/or function in tumor and tumor-associated cells, which promotes antitumor activity and synergizes with anti-PD-1 therapy. In combination with chemotherapy and anti-PD-1 therapy, restoration of PDLIM2 has demonstrated complete remission of most animals with lung tumors, establishing a new foundation for PDLIM2-based combination therapies for cancer treatment. Additionally, PDLIM2 expression and function in tumor cells and tumor-associated cells can be used as a marker to assess cancer risk, diagnosis, prognosis and treatment response.	Oncology		Antibody,protein,peptide	Protein	Qu	Zhaoxia	Med-Microbiology and Molecular Genetics	In vivo data	License
4566	Oncolytic Viruses Expressing Cytokine IL-36γ for Cancer Therapy	Novel oncolytic vaccinia viruses were constructed to express the secreted form of IL-36γ. The virus infects cancer cells, induces oncolysis, and secretes the cytokine from the infected cancer cells. The addition of IL-36γ enhances the antitumor activities of the oncolytic viruses by promoting an adaptive T cell-mediated immune response and stimulating the immunogenic tumor microenvironment. In models of colon cancer, pancreatic cancer, and melanoma, direct injection of the armed virus led to superior antitumor effects.	Oncology		Drug delivery		Guo	Zongsheng	Med-Microbiology and Molecular Genetics	In vivo data	License
4518	Targeting Highly Tumor-specific Long Non-coding RNAs for Cancer Diagnosis, Prognosis, and Therapy	Investigators at the University of Pittsburgh have identified a group of cancer-related lncRNAs as novel biomarkers of cancer. In addition, they developed methods of detecting and inhibiting these molecules in cancer cells. Researchers paired the detection of these lncRNAs with genetic and clinical data from 1,023 breast tumor samples and 24 breast cancer cell lines. By integrating the lncRNA profile with clinical outcome data, investigators have concluded that these lncRNAs are important players of tumorigenesis and clinical prognosis. Among the 2,123 lncRNAs identified, one in particular appears to have higher expression in nine different cancer types including breast cancer. Inhibition of these lncRNA in breast cancer cells led to cell death, suggesting therapeutic potential in treating breast cancer.	Oncology		Antisense,RNA		Yang	Da	Pharm-Pharmaceutical Science	In vitro data	License
4461	NKCC Inhibitors for Neuroprotection Following a Stroke	To overcome the limitations of bumetanide as a stroke treatment,researchers havedeveloped lipophilic and uncharged bumetanide derivatives thatpenetrate theblood–brain barrier more easily. Changes to the structure of the bumetanide molecule could also curb diuresis by conferring greater selectivity for NKCC1 —which is primarily expressed in the brain —over NKCC2 in the kidney. In a mouse model of stroke, one of the new compounds, STS66, was more effective than bumetanide at reducing cell death, swelling, and neurological deficitsin the weeks after the ischemic event. The mice receiving STS66 even lived longer.	Neuroscience		Small molecule		Sun	Dandan	Med-Neurology	In vivo data	License
4454	Approaches to Counteract Age-related Cognitive Decline	Researchers at the University of Pittsburgh have discovered that certain exercise-induced circulating factors may serve as useful biomarkers to detect the efficacy of a rehabilitation program to counteract age-related declines in tissue and organ function. For instance, one can expect that different programs (e.g., aerobic exercise, resistance exercise, yoga/mindfulness or health education) will have different effects on the structural and functional cognitive outcomes in aged individuals. The early identification is advantageous, as it may allow clinicians to determine early on whether a program is effective and enables the program to be tailored to the patient. The same research group has also developed a novel technology—exosome engineering and transplantation—for delivery of Klotho transcripts, messages to promote muscle and brain health. This important finding can reveal the specific therapeutic signals that drive Klotho anti-aging effects.	Musculoskeletal	Neuroscience	Antibody,protein,peptide	Aging; Peptide; Synthetic Biology	Ambrosio	Fabrisia	Med-Physical Medicine & Rehabilitation	In vivo data	License

4453	A Novel Multifunctional Drug Delivery System for Chemo-Gene Combination Therapy	The present invention is a novel micellar system composed of cationic amphiphilic polymers for co-delivery of small molecule chemotherapy drugs and therapeutic genes. Researchers at the University of Pittsburgh have developed novel polymeric carriers composed of PEG hydrophilic segments and cationic moieties. These polymers have the ability to form micelles, which can effectively load hydrophobic drugs while simultaneously forming complexes with nucleic acids. When co-loaded with a drug and plasmid DNA, these micelles are observed to be significantly smaller and more stable than particles loaded with the drug alone. In this system, the multivalent charge–charge interactions between the cationic polymer and plasmid DNA serve as a simple approach to cross-link the micelles, thus making these micelles more stable than free micelles or micelles loaded with small molecule alone. As a working example, investigators developed a polymer for co-delivery of IL-36γ expression plasmid and doxorubicin (Dox) to lung metastasis of breast cancer. The use of this polymer resulted in significantly higher gene transfection in both lungs and tumors compared to control and, in addition to this improved anti-metastatic effect, synergistically enhanced the type I immune response and decreased immunosuppressive cells in the lung.	Oncology		Drug delivery	Drug delivery	Li	Song	Pharm-Pharmaceutical Science	In vivo, mice	License
4368	YouBiotic	YouBiotics are created from an individual's own bacteria which facilitates engraftment in the small intestine and improves tolerance. Conventional probiotics can produce gastrointestinal discomfort and do not colonize the intestines. We have tested YouBiotics in a set of rigorous metabolic cage experiments in mice. Our data indicates that YouBiotics do not alter the animal's metabolism, and that the weight changes are not a result of water loss, increased activity, or loss of lean mass. Taken together, YouBiotics act to scavenge dietary fats from the intestines beforethey can enter the blood stream. In this way, dietary calories from fat can be significantly reduced, facilitating reductions in fat mass and blood triglycerides with long-term use. Because YouBiotics is a probiotic, we intend to market it as a food supplement reducing carry-over costs and development time associated with FDA approval. Our current manufacturing cost estimates indicate that YouBiotics can be a low-cost weight management therapy with a price point of ~$99-$199for a one-month supply, which represents as much as 93% cost reduction in comparison to FDA regulated weight loss therapeutics.	Other	Other	Other		Acharya	Abhinav	Chem/Petroleum Engineering	Completed: in vivo experimentation in small animals In progress: preclinical study in large animal model	License;NewCo
4358	Preserving Harvested Fat Between Grafting Procedures	The multi-functional vessel is a 10cc chamber with Luer Lock ports on both ends and a built-in filtration system. After harvesting fat graft material, the vessel interfaces with the harvesting syringe for easy transfer. Then cryo-protectant solution is added to the vessel and it storedin a hospital freezer. When necessary, thawing and washing occurs in the same closed system of the storage vessel. Currently, onlyone tissue banking company that offers off-site cryo-storage of adipose tissue grafts,andshipping the material back and forth is expensive and complicates procedure scheduling. Because our system involves storing the tissue on-site, it enables multiple treatments with minimal additional costs after the original fat harvest and processing.	Plastics		Medical Device		Rubin	Joseph	Med-Plastic Surgery	Prototype	License;NewCo
4279	Gene Therapy for Male Infertility without Germline Transmission	To establish the proof-in-principle for testicular somatic cell defects, we designed an adenovirus (Ad) vector to introduce a therapeutic human androgen receptor (hAR) gene into an AR-deficient mouse model of human NOA. Ad-hAR injections restored spermatogenesis in 90 percent of seminiferous tubules in the testes. Histology in these mice showed that Ad-hAR transfects only Sertoli cells — somatic cells that facilitate spermatogenesis— and not the sperm or sperm producing cells. As a result, none of the treated males' progeny carried the transgene. In parallel, we devised a strategy for ex vivo gene editing (using CRISPR/Cas9) followed by transplantation of germline stem cells. In the case of homozygous recessive disease, we outline how this approach can be deployed for germline gene therapy without germline transmission to progeny. For men faced with one of the most intractable types of infertility, our gene therapy method offers a potential cure without the ethical concerns of germline transmission. As societal concerns about germline gene editing evolve, the technologies described here can be used to purposefully eliminate the world's most devastating diseases from families.	Urology		Gene therapy		Orwig	Kyle	Med-OB-Gyn & Reproductive Science	In vivo data	License
4267	Ribosomal Protein-Based Diagnostic and Prognostic Test for Cancer	To identify and classify RP transcript patterns, we applied an advanced form of machine learning called T-distributed stochastic neighbor embedding (T-SNE) that uses a variety of linear and non-linear relationships to cluster data. When applied to human tissue data from the cancer genome atlas, this method was 93%accurate at distinguishing between tissue types and more than 98%accurate at discriminating tumors from normal tissue. In at least ten different common tumors typesincluding hepatocellular carcinoma, kidney, brain and endometrial cancer,the pattern of RP transcripts was also highly predictive of survival. Our proprietary T-SNE-based RP transcript analysis program could form a clinically useful bioinformatics platform to accurately determine a tumor's tissue of origin, classify known tumors into subtypes, and stratify patients into high-andlow-risk categories. This information will be useful for determining the most appropriate treatment plan for individual patients.	Oncology		Molecular diagnostic		Prochownik	Edward	Med-Pediatrics	Software	License
4250	Novel Glycine Receptor Modulators for Analgesia	Recognizing that glycine receptors are responsible for the analgesic effects of marijuana, we screened a library of drug-like molecules for structural compatibility with the same glycine receptor binding site as THC. A representative compound from this group – ZINC08 – was even more effective than THC at enhancing human glycine receptor function in vitro. In mouse behavioral tests, ZINC08 reduced the effects of inflammatory pain and boosted the efficacy of a sub-therapeutic dose of morphine. Patients and prescribers could use ZINC08 and other glycine receptor modulators in its class to reduce the necessary dose of opioids for pain management, eliminating side effects such as dependence, tolerance, addiction, sedation, and nausea.	Central Nervous System		Small molecule		Xu	Yan	Med-Anesthesiology and Perioperative Medicine	In vitro and in vivo behavioral data	License
4181	Chemical Pancreatectomy Using Ethanol Infusion	By one theory of chronic pancreatitis, the exocrine pancreas, which produces digestive enzymes, creates a toxic environment that then kills off the otherwise healthy insulin-producing islets of the endocrine pancreas. We discovered that infusing pure ethanol into the pancreatic duct of a mouse leads to complete destruction of the problematic exocrine pancreas while leaving the endocrine pancreas intact. In a model of chronic pancreatitis, ethanol infusion halted pancreatic islet destruction and improved insulin production. As opposed to a traditional pancreatectomy, our method can be performed endoscopically for minimal invasiveness. Also, because ethanol infusion spares the hormonal functions of the pancreas, our method could treat the painful and carcinogenic aspects of pancreatitis while also reducing patients' risk of developing diabetes.	Endocrinology		Other		Gittes	George	Med-Surgery	In vivo mouse data, in situ primate surgical protocol	License
4169	ThreadRite IV	ThreadRite is a modified standard catheter that immediately alerts clinicians to vein entry. Consisting of a modified standard IV cather connected to a lightweight reusable detection unit, ThreadRite employs a guidewire to help clinicians insert the IV properly on the initial attempt. Once in the vessel, the guidewire aids in catheter advancement. ThreadRite has the potential to reduce patient pain and lower provider costs associated with this incredibly common problem.	Cardiovascular		Medical Device		Dezfulian	Cameron	Med-Critical Care Medicine	The research has progressed to testing of the sensor in pigs, and the team is planning an initial human for the end of 2021/beginning of 2022. The team has also made good	License
4124	Targeting Genetic Changes in IDH-Mutant Gliomas with a Novel Use of an FDA-Approved Drug	Our novel use of the drug works by reversing the immune-evasive properties of IDH-mutant gliomas. The immunotherapeutic properties of this compound are due to activation of immune target receptors specifically in cancer cells, rendering them significantly more susceptible to killing by NK cells and T cells. In addition, treatmentreduces tumor size and growth by modulating IDH-mutant cancer cell death and differentiation. Compared to current broad-spectrum treatments like tumor resection, radiation, and chemotherapy, our genetically targeted immunotherapeutic approach may avert tumor recurrence.	Oncology		Small molecule	Small molecule	Amankulor	Ndukaku	Med-Neurological Surgery	In vivo mouse and human data	License
4085	Targeting Chromosomal Rearrangements for Treatment of Cancer	The present invention relates to methods of detecting and treating patients suffering from cancer or a pre-malignant or neoplastic condition and are carrying one or more specific fusion genes. These specific fusion genes yield insights into disease progression and enable clinicians to tailor specific genome therapies. The chromosomal breakpoints of significant numbers of fusion genes have been identified; these breakpoints not only serve as cancer markers but also provide unique opportunities to treat human cancers using genome editing and genome targeting technologies. Once fusion transcripts are detected in serum samples, novel treatment options include administering a therapeutic effective amount of an agent at the breakpoint that inhibits the fusion gene of interest.	Oncology		Other diagnostic		Luo	Jianhua	Med-Pathology	In vivo data	License
4062	Tetrahydrocannabinol (THC) Sensor for a Marijuana Breathalyzer	Pitt researchers have developed a novel tetrahydrocannabinol (THC) sensor using single walled carbon nanotubes (SWCNTs) to quantify THC dissolved in ethanol or in a dried state. The sensor can be implemented in a handheld breathalyzer to quantifiably measure THC in someone who is under the influence of marijuana. Compared to competing THC breathalyzer technologies, which are expensive or involve complicated multistep processes, this sensor boasts high sensitivity, low power consumption, and low fabrication costs.	Other		Medical Device		Star	Alexander	Chemistry	Concept	License
4054	Reversing Fosfomycin Resistance	Researchers at the University of Pittsburgh have discovered a compound called ANY1 that selectively blocks the mechanism by which bacteria resist fosfomycin treatment. When applied together with ANY1, fosfomycin is once again effective against formerly resistant bacteria. Bacteria mount resistance by deploying fosfomycin-modifying enzymes, most frequently FosA. ANY1 restores fosfomycin sensitivity in these pathogens by competitively inhibiting FosA. In control experiments, ANY1 did not have any impact on bacterial growth when applied alone, nor was it efficacious in conjunction with fosfomycin against a strain of E. coli that was engineered to lack the FosA gene. This cocktail presents the opportunity to fight back against so-called superbugs that evade all known current treatments.	Infectious Disease		Small molecule		Sluis-Cremer	Nicolas	Med-Medicine	In vitro data	License
4010	Rapid Response Preventative Vaccines for Zika Virus Outbreak	Pitt researchers have developed a recombinant adenoviral vector-expressing codon-optimized subunit recombinant ZIKV E vaccine combined with a skin-targeting vaccine delivery technology to specifically create advantages in immunogenicity, economy, and safety in order to enable broad, effective clinical deployment. The vaccine delivery strategy utilizes an intracutaneous microneedle array (MNA)-based delivery system, found to be superior to intramuscular administration in both the potency and duration of the induced immune response. The MNA vaccine delivery system also affords unique advantages in reproducibility, safety, manufacturing, and distribution, by relieving pressure on the cost-intensive "cold chain" required to preserve vaccine potency within a restricted temperature range for delivery and distribution in developing countries. The synergistic integration of this effective vaccine and delivery method has distinct advantages critical for widespread clinical deployment.	Vaccines		Vaccine		Gambotto	Andrea	Med-Surgery	In vivo data	License

3916	Mitoparib: A Mitochondrial PARP Inhibitor that Counteracts Metabolic Stress	To hone in on mitochondrial PARP, we added a targeting sequence to the PARP-inhibitor veliparib, which is currently in multiple late-stage clinical trials for cancer, to create mitoparib. Whereas veliparib fights cancer by hastening DNA damage to invoke cell death, mitoparib protects environmentally-stressed cells against cell death by inhibiting PARP-related NAD+ depletion in the mitochondria while permitting homeostatic PARP-related DNA repair in the nucleus. Experiments with oxygen- and glucose-deprived rat neurons demonstrate that mitoparib reduces mitochondrial PARP activity and ameliorates mitochondrial swelling associated with necrosis. In mouse embryos, mitoparib decreases radiation-induced cell death. Mitoparib presents an exciting solution to targeting mitochondrial PARP activity and prevent cell death.	Oncology	Small molecule		Wipf	Peter	Chemistry	In vivo data	License
3868	Oncolytic Viruses Armed with Membrane-Associated Immunomodulatory Molecules for Cancer Therapy	Oncolytic vaccinia viruses armed with a gene encoding a membrane-associated fusion protein that includes an immunomodulatory molecule can be used to deliver membrane-associated immunomodulatory molecules to the tumor site. Rather than simply lysing the tumor cells and/or secreting the cytokine from the cancer cell, these cytokines are expressed on the cancer cell membrane. This helps to deliver cancer-fighting compounds to areas where they are most needed as well as reducing possible adverse toxicity by avoiding systemic diffusion. Candidate immunomodulatory molecules for this delivery method include cytokines, such as cytokine IL-2, and natural or man-made protein domains and peptides. This method can be used to achieve enhanced anti-tumor immunity and therapeutic effects, and has been demonstrated on a colon cancer model using an oncolytic vaccinia virus expressing membrane-associated fusion protein IL-2-GPI.	Oncology	Drug delivery	Immuno-oncology	Bartlett	David	Med-Surgery	In vivo data	License
3854	Compounds that Convert Acinar Cells into Insulin-Producing Beta Cells	Researchers at the University of Pittsburgh have discovered a compound which, when administered to diabetic mice or non-human primates (NHP) in therapeutic amounts, has the potential to normalize blood glucose by transforming pancreatic acinar cells into beta cells. These acinar-derived beta cells migrate into and embed themselves within the microenvironment of the islets of Langerhans, conferring distinct advantages beyond insulin production, including proximity to blood vessels that provide for increased efficiency of insulin secretion. The acinar-derived insulin-positive cells also express Glut2, suggesting that these cells share features with mature insulin-producing cells.	Endocrinology	Small molecule	Small molecule	Esni	Farzad	Med-Surgery	In vivo data	License
3811	p97 ATPase Inhibitors for Cancer and Neurodegeneration	Structural analysis indicates that these compounds bind allosterically to act literally as a "wrench in the works", blocking the motion of the p97 protein subunits. Inhibition of p97 ATPase activity triggers downstream cellular effects, including the suppression of cellular proliferation that makes cancer such a dangerous disease. And because these compounds specifically inhibit p97, they offer the possibility of treating a wide array of ailments with fewer side effects than drugs acting on broader pathways; neurodegenerative diseases, for example, display dysregulation of protein homeostasis via p97 and have the potential to be treated with these inhibitors.	Neuroscience	Small molecule		Huryn	Donna	Pharm-Pharmaceutical Science	In vivo data	License
3624	Compounds Targeting Androgen Receptor to Treat Castration-Resistant Prostate Cancer	Innovators at the University of Pittsburgh have identified novel small molecules that bind to the androgen receptor (AR) and block the nuclear localization and function of AR in CRPC cells. The compounds are not cytotoxic and decrease nuclear AR levels in CRPC cells. Xenograft studies using these small molecules showed inhibition of castration-resistant growth of C4-2 and 22Rv1 xenograft tumors in SCID mice. This work demonstrates the potential of these compounds in CRPC tumor therapy. A second class of compounds significantly decreases cell proliferation in AR-positive cell lines while they have no effect on proliferation in AR-negative cell lines.	Oncology	Small molecule	Anti-Androgens	Wipf	Peter	Chemistry	In vivo and In vitro data	License
3620	CardioSense	CardioSense is a handheld, multi-array biosensor that screens for cardiovascular risk by detecting FDA-approved cardiac markers in just a few drops of blood, similar to the function of a glucose tester. CardioSense screens for cardiac markers using aptamers, which are single-stranded nucleotide sequences that are synthesized to bind targets with high sensitivity and specificity. Two targets of interest for CVD are brain natriuretic peptide and Troponin-T, which are indicators of cardiac stress or injury. Aptamer binding is detected using an impedimetric device, allowing for a quick, simple, and cost-effective mode of detection compared with blood assays (e.g., ELISA) or imaging tests (e.g., CT) conducted in the hospital and diagnostic laboratories.	Cardiovascular	Medical Device		Kumta	Prashant	Bioengineering	Optimization in the laboratory	License
3178	A Simple, Effective, and Dual-Functional Drug Delivery Platform for Hydrophobic Agents	A novel drug formulation based on PEGylated FTS, a synthetic farnesylcysteine mimetic, acts as a potent and particularly non-toxic antagonist of the Ras family proto-oncogenes present in one-third of human cancers. FTS inhibits the growth of Ras-dependent tumors with no significant toxicity; in addition to its antitumor activity in mice and humans, FTS also exhibits anti-inflammatory activity. PEGylation serves to improve the solubility of FTS, which has a hydrophobic nature and limited bioavailability. The PEG-FTS conjugate forms small-sized micelles, a type of delivery system that has gained considerable attention due to micelles' small size and ability to solubilize water-insoluble anticancer drugs and accumulate specifically at tumor sites. In this new formulation, both the anticancer drug and the PEG-FTS carrier display antitumor activity and can synergize to amplify the effect. Further, the drug-loading capacity and formulaic stability of the PEG-FTS micellar system can be further improved via incorporation of a drug-interaction motif, leading to the creation and development of highly effective therapeutics with minimal toxicity at a low cost in a timely manner.	Oncology	Small molecule		Li	Song	Pharm-Pharmaceutical Science	In vivo data	License
3007	SimultaneousInhibition of Wnt, TGF-beta and Hippo Signaling to Treat Cancer, Organ Fibrosis and Neuropathic Pain	Building on knowledge of cancer cell reprogramming, researchers have identified a pipeline of novel anticancer agents with dual action on cell proliferation and EMT. C19 is a promising small molecule candidate with remarkable inhibitor activity against Hippo, Wnt and TGF-beta pathways: it induces TAZ degradation through activation (phosphorylation) of Hippo kinases MST/LATS and the tumor suppressor kinase AMPK, which is an upstream regulator of the degradation complex of YAP. It has been demonstrated that C19 inhibits cancer cell migration, proliferation, and resistance to doxorubicin in vitro, and exerts strong anti-tumor activity in mouse tumor models. By simultaneously targeting multiple EMT pathways, this novel compound provides a new class of agents that have the potential to not only suppress cancer progression but also prevent its recurrence.	Oncology	Small molecule	Small molecule	Rebbaa	Abdelhadi	Med-Pathology	In vivo data	License
2659	An Oral Candidate Prevention Therapyfor Melanoma	Sulforaphane (SFN) is a naturally occurring compound found in vegetables such as broccoli, cabbage, and cauliflower, among many others. SFN is renowned for its anti-cancer properties and is easily isolated from natural sources. Using our identified formulation and dosage, SFN effectively delayed melanoma progression in mice. Additionally, we identified possible biomarkers for the progression of atypical nevi to melanoma.	Oncology	Small molecule		Kirkwood	John	Med-Medicine	In vivo data	License
2631	Global Influenza Vaccine Discovery Uses Computational Approach that Shows Promise Preventing Multiple Strains	Pitt researchers have found a solution by developing a recombinant tetravalent HA-DBV vaccine, which holds potential for broad efficacy in the prevention of influenza. The HA-DBV vaccine simultaneously displays immunogenic proteins derived from four subclades of H5N1 influenza viruses. It offers robust protection against influenza infection in vivo, generating both cellular and humoral immune responses. To generate their candidates, investigators assessed the efficiency of a computationally optimized influenza hemagglutinin (HA) protein. They found that the signal peptide (SP) and cytoplasmic tail (CT) domains of gp64 can enhance the display of HA, while the transmembrane domain of gp64 impairs HA display. By constructing a recombinant proteins, researchers discovered a method that computationally optimizes the influenza HA protein to generate immunity against a broad range of clades within each influenza subtype, including H1N1, H3N2, H2N2, and B influenza. This technology generates HA sequences with high immunogenic activity that results in cross-reactive immune responses to all viruses tested using a single HA sequence. The discovery suggests both humoral and cellular immunity are robustly generated against a broad range of influenza clades. Scientists and researchers are developing additional vaccines using this same computational method, which may lead to vaccine development for other infectious diseases.	Infectious Disease	Vaccine	Computational Vaccine Development; Respiratory Viruses	Ross	Ted	Department of Infectious Diseases	In vivo and In vitro data	License
2522	ResMag: A Resorbable Magnesium Alloy for Medical Applications	To improve the success rate of orthopedic implants and reduce the use of permanent stainless steel or titanium implants, researchers at the University of Pittsburgh have developed ResMag, a novel resorbable magnesium alloy. Magnesium is a preferred material because it is non-toxic, degrades in a physiological environment, is lightweight, has a density similar to cortical bone, has elastic modulus that is much closer to natural bone, is essential to human metabolism, is a cofactor for many enzymes, and stabilizes the structures of DNA and RNA. The novel biodegradable, metal alloy is comprised of about 0.5 to about 4.0 weight percent of yttrium, from greater than zero to about 1.0 weight percent of calcium, from about 0.25 weight percent to about 1.0 weight percent of zirconium, and a balance of magnesium, based on the total weight of the composition. The ResMag technology has been successfully tested in cellular assays and in rodent subcutaneous, osteotomy and cranial models implanting the alloy subcutaneously as well as in fractured femurs, and calvaria. In all the rodent studies, ResMag fostered a significantly higher rate of cell viability and did produce gas pockets within a week of implantation that subsided in time. There was no accumulation of magnesium or other metals in the blood or organs, and no measurable adverse effects of the ResMag implant systemically in the blood, serum, liver, spleen, kidney, brain or within and around the implanted tissue.	Orthopedics	Biomaterial	Biomaterial; Regenerative medicine	Kumta	Prashant	Bioengineering	Demonstrated in cellular assays and limited animal models	License