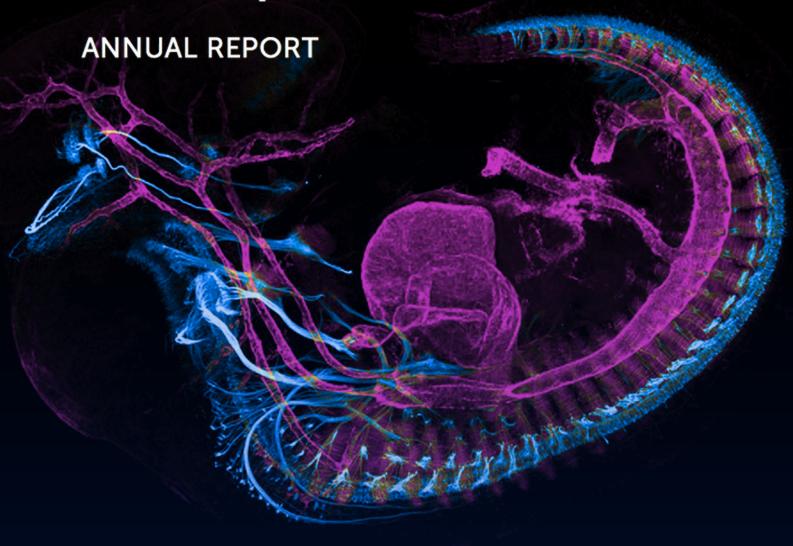
2023

Technology & Innovation Development Office

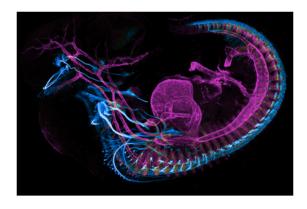




TIDO in

FY23

RESEARCH EXPENDITURES	\$550.9M
NEW INDUSTRY-SPONSORED RESEARCH FUNDING	\$15.2M
RESEARCH AGREEMENTS	57
LICENSING AGREEMENTS	46
GROSS LICENSING REVENUE	\$8.7 M



On the cover

Developing Mouse Embryo

A developing mouse embryo showing muscles and motor neurons. (Color edited, muscles in magenta, motor neurons in blue) Jessica Bell, BS, and Mary Whitman, MD, PhD Department of Ophthalmology Boston Children's Hospital

From the Director

2023, A YEAR OF GROWING IMPACT ON INNOVATION AT BOSTON CHILDREN'S HOSPITAL.

In 2023, we saw initial signs of modest recovery in our life science sector. It was one of the strongest over the last 5 years in terms of FDA approvals with 55 new drugs and 25 biologics[1], close to \$20B raised by US VC firms for health-focused funds[2], and robust M&A activity with many pharma companies acquiring biotech companies mainly at the clinical trial stage[3]. However, VC funders remained cautious making the environment challenging for earlier stage biotech companies. Fortunately, U.S. government funding focused on R&D continued to increase with a 5-year growth in NIH funding of 35% compared to the 17% growth in the previous 2012-2016 period[4].

This context clearly shows the importance of identifying and nurturing the next therapeutic and medical device innovations to draw interest from future strategic partners for a clear impact on patient lives. At the Technology and Innovation Development Office (TIDO), we increased delivery on our promise in this matter. We recruited Dr. Nadine Beauger, Senior Director of our new Therapeutic and Medical Device Accelerator (the "Accelerator"). This exciting new initiative will expedite the translation of promising therapeutics from the laboratory to clinical applications through significant project funding, mentorship, and access to industry partners.

At TIDO, we take pride in harnessing the problem-solving mindset of our BCH investigators to make a positive impact on patient lives. Our dedicated team continued to work diligently to advance the mission of translating research into transformative products and therapies as we remained focused on our goal. We are thrilled to share that we secured new licenses and sponsored research agreements with an array of partners from a spin-off company (ClearCut Surgical Inc.) to multi-national pharma partners (Novo Nordisk, Merck, Pfizer), through specialized biotech companies (EveryONE Medicines) and public-private partnerships (Northpond Lab Alliance-Wyss Institute). Those agreements spanned the different areas of expertise of our BCH investigators, namely a medical device developed by and for surgeons, new treatments for rare diseases, new adjuvants for more effective vaccines, and a screening platform to identify novel therapeutic compounds. With our missing of making a true impact on patient lives, we are also thrilled to report positive Phase 2 trial data from Scholar Rock, one of our spinoff companies by Drs. Timothy Springer and Leonard Zon. Even in those challenging VC funding times, Miach Orthopedics, founded by Dr. Martha Murray, managed to secure a \$40 million financing allowing the launch of the BEAR implant study. This implant treats ACL tears, which are one of the most common knee injuries. It was developed at Boston Children's Hospital with initial research funding provided by the NFL Players Association, the National Institutes of Health, and a TDF (Technology Development Fund) grant. As highlighted in the report, we continued to support further projects through TDF and D3A to help bring a next wave of innovative solutions closer to the patients.

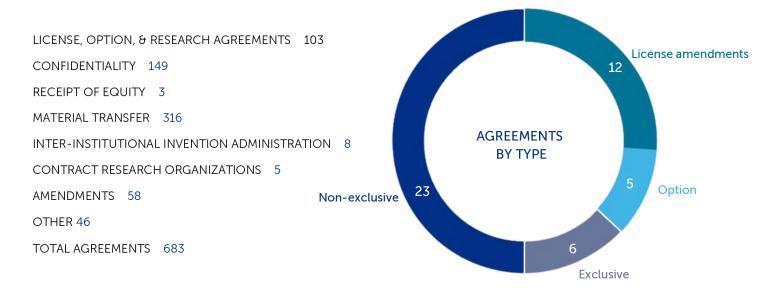
I want to take this opportunity to thank our TIDO team for their relentless support of our BCH Faculty members, bringing their complementary expertise and know-how to each project they worked on. Our continued successes were made possible through the unwavering confidence of our internal and external partners. We are confident that TIDO will further advance future BCH innovations as we continue our mission of translating cutting-edge research into real-world solutions, creating a positive impact on human health and well-being.



Irene AbramsVice President, Technology Development and New Ventures
Technology & Innovation Development Office

- [1] Novel Drug Approvals for 2023 | FDA
- [2] Healthcare Industry Trends | Silicon Valley Bank (svb.com)
- [3] Trends in Life Sciences February 2024 Life Sciences Update (cushmanwakefield.com)
- [4] Global Funding February 2024 Life Sciences Update (cushmanwakefield.com)

Agreements



Impact

ACADEMIC PARTNERSHIPS 254

INDUSTRY PARTNERS 233

CORPORATE-SPONSORED RESEARCH & COLLABORATIONS 57

STARTUPS CREATED 3

Revenue

REVENUE FROM FY23 LICENSES AND OPTIONS \$7.879,102

NEW INDUSTRY SPONSORED RESEARCH FUNDING \$15,206,988

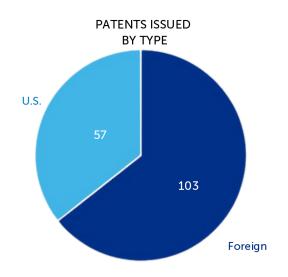
GROSS REVENUE \$8,729,102

Intellectual Property

NEW DISCLOSURES 177

PATENTS ISSUED 160

PATENT APPLICATIONS FILED 294



Licences & Sponsored Research



ClearCut Surgical exclusively licenses electrocautery device

ClearCut Surgical Inc., a company founded by surgeons and specializing in the production of innovative surgical devices, has entered into an exclusive license agreement with Boston Children's Hospital for the development and production of an innovative electrocautery device that was invented by Heung Bae Kim, MD, the Director of the Pediatric Transplant Center at Boston Children's Hospital. Electrocautery is one of the most commonly used surgical devices in the majority of specialties. Tissue dissection using electrocautery results in the creation of surgical smoke as well as the accumulation of fluid and blood that requires frequent removal. While devices currently exist for removal of surgical smoke, no current electrocautery devices allow for the removal of fluid or blood. Dr. Kim and his team have developed a new device that not only removes surgical smoke but also toggles quickly with the push of a button to become a suction device allowing for efficient removal of blood and fluids. This device allows the surgeon to control fluid evacuation easily and quickly, improving efficiency and potentially improving outcomes while decreasing costs due to the time savings.



EveryONE Medicines sponsors research for ataxiatelangiectasia

EveryONE Medicines, a therapeutic biotechnology company delivering individualized precision therapeutics customized to the unique pathogenic genetic mutation of a single or a very small number of patients, has sponsored research from the lab of Timothy Yu, MD, PhD, in the division of Genetics and Genomics to research Ataxia-Telangiectasia (A-T). A-T, also known as Louis-Bar syndrome, is a rare inherited childhood neurological disorder that affects the part of the brain that controls motor movement and speech. It currently has no cure and treatment options are limited to supportive care. The research is focusing on the clinical exploration of atipeksen, an investigational, mutation-specific antisense oligonucleotide (ASO) therapeutic. ASO therapies, which target RNA, are particularly promising as modality is well understood, can precisely target certain mutations, and is known to show good uptake in the brain. The sponsored research will also include development and qualification of hybridization ELISA (enzyme-linked immunosorbent assay) methods to measure ASO drug levels in human cerebrospinal fluid (CSF) and plasma samples, which will inform clinical dosing. Most importantly, the main goal is to develop further ASOs to treat other mutations causing A-T.



Novo Nordisk sponsors research for X-linked sideroblastic anemia

Novo Nordisk, a company developing treatments for people living with diabetes, obesity, and rare blood and endocrine disorders, has sponsored research from the lab of Dr. Mark Fleming, MD, DPhil, in the Department of Pathology, evaluating the implementation of a new therapy for X-linked sideroblastic anemia (XLSA), the most common inherited form of Sideroblastic Anemia.

XLSA is a congenital form of anemia but can manifest at any age in both males and females. Its principal symptoms result from reduced hemoglobin (anemia), but the disease can be complicated by iron overload, which can lead to liver cirrhosis and heart failure, and even ultimately death. The novel approach being investigated employs a unique mouse model of XLSA developed in the Fleming laboratory.



Merck sponsors research for adjuvants against SARS-COV-2

Merck & Co, Inc., a US headquartered, global biopharmaceutical company, has sponsored research to evaluate vaccine adjuvants with cross-species and age activity under the direction of Ofer Levy, PhD, Director of the Precision Vaccines Program (PVP) at Boston Children's Hospital. Adjuvants, which are added to vaccines to enhance the body's immune response, are a promising avenue of research for the creation of more effective vaccines against all indications. As part of the research, the PVP will model adjuvant-induced innate immune activation in human neonates, young adults, and older adults in vitro by performing human leukocyte assays. The PVP will also do in vivo studies to investigate immunity against SARS-CoV-2 wildtype spike protein and RSV F protein in mice.

Licences & Sponsored Research



Wyss Institute and Northpond Labs sponsor research for therapeutic-discovery platform for nanoswitch screening

The Wyss Institute at Harvard University uses biologically inspired engineering principles to develop new engineering innovations to transform healthcare. In 2020, the Wyss Institute formed an alliance with Northpond Labs to support early-stage, transformative research with strong potential. As a result, Boston Children's Hospital (BCH), Northpond Labs and the Wyss Institute are participating in a program in which Northpond has committed to select and fund research projects designed and performed by researchers at the Wyss Institute and BCH. Northpond Labs has selected to fund principal investigator Wesley Wong, PhD, in the Program in Cellular and Molecular Medicine for a next-gen nanoswitch therapeutic-discovery platform for compound screening.

The Wong Lab will develop DNA nanoswitches capable of screening for potential therapeutic compounds.

Nanoswitches are nanoscale tools that can report molecular interactions by changing shape, enabling interaction measurements using inexpensive and common laboratory reagents. The Wong lab intends to screen for compounds that can modulate protein-protein interactions that govern a variety of conditions, including autoimmune disorders and cancer. Although there are many tools and methods to find compounds that disrupt protein-protein interactions, there remains a need for methods to identify molecules that promote these kinds of interactions. Nanoswitches, which are easy to use and affordable, can provide vital screening and measurements to identify potential therapeutic compounds.



Pfizer sponsors research into neutrophil phenotypes

Pfizer Inc., a multinational pharmaceutical company headquartered in Delaware, has partnered with Peter Nigrovic, MD, Chief of Immunology at Boston Children's Hospital, and the Grieshaber-Bouyer lab at Friedrich Alexander University in Erlangen, Germany, to conduct an in-depth evaluation and characterization of different subsets of neutrophils, in both healthy individuals and those with inflammatory diseases. Neutrophils play a crucial role in immune defense and the regulation of inflammatory responses. In inflammatory conditions such as arthritis, dysregulation of neutrophil immune responses can lead to disease initiation and perpetuation.

Previous work in the Nigrovic lab has shown that depleting or halting the migration of neutrophils can help arrest deregulated inflammatory responses in arthritis. However, the current project aims to go further by targeting specific neutrophil states or phenotypes that are specifically involved in inflammatory dysregulation. To achieve this, the project will involve characterizing blood and bone marrow neutrophils from both healthy and non-healthy donors. This characterization will include quantifying cell surface proteins and analyzing transcriptomic data from the same cells. The goal is to gain a comprehensive understanding of these different neutrophil subsets and their potential as therapeutic targets for inflammatory diseases.

Milestones



Miach Orthopedics secures \$40 million financing and launches BEAR Implant registry study

Miach Orthopedics, founded by Martha Murray, MD, in the Department of Orthopedic Surgery, announced the closing of a \$40 million financing round. The funds will enable ongoing operations and expand the U.S. commercial rollout of the BEAR Implant. The BEAR implant was developed at Boston Children's Hospital with initial research funding provided by the NFL Players Association, the National Institutes of Health, and a TDF (Technology Development Fund) grant. The implant treats ACL tears, which are one of the most common knee injuries. Prior to the BEAR implant, ACL tears were treated with surgical reconstruction, which involved removing a torn ligament and replacing it with a graft of a tendon from elsewhere in the body or from a cadaver. Although effective, ACL reconstructions often led to early arthritis or other complications. Dr. Murray and her team pioneered the development of a scaffold made of collagen and other extracellular matrix proteins that allows the patient to regrow their own ACL. The company also announced the initiation of the Bridge Registry Post-Market Study of the BEAR Implant to track outcomes in patients who have received the implant to supplement the base of clinical trial research that has already been completed.



Scholar Rock announces positive results in Phase 2 trials for apitegromab to treat spinal muscular atrophy

Scholar Rock announced data from the Phase 2 TOPAZ trial extension period evaluating patient outcomes at 36 months of treatment with apitegromab, a selective inhibitor of the activation of latent myostatin. Results showed that continued treatment with apitegromab over the extended treatment period was associated with substantial and sustained improvement in motor function, as well as improvements in patient-reported outcome measures in patients with nonambulatory Types 2 and 3 spinal muscular atrophy (SMA) receiving survival motor neuron (SMN)-targeted therapy. Scholar Rock was co-founded by Timothy Springer, PhD, an investigator in the Program in Cellular and Molecular Medicine, and Leonard Zon, MD, Director of the Stem Cell Research Program.

Strategic Alliances

TIDO's mission is to advance novel therapeutics developed at Boston Children's Hospital from the laboratory bench to the patient's bedside. To support this vision, we have several multi-year strategic alliances with industry partners to significantly reduce the administrative and resource hurdles that can hinder collaborative scientific progress. Our partners include:



















FY23 Alliance Team







Michael Bergin, PhD, MBA Strategic Alliance Manager

FY23 Alliance News



Autobahn Labs investigates AZIN1 in target validation campaign

Autobahn Labs is a drug discovery accelerator that partners with academics to de-risk novel research and develop transformational new medicines. As a strategic partner to BCH, Autobahn is evaluating Antizyme Inhibitor (AZIN1) as a potential cancer target in collaboration with Drs. Bruce Zetter, PhD, and Michael Rogers, PhD. Work from their labs, and others, suggests that inhibiting AZIN1's binding to antizyme may be a novel approach to suppressing cancer growth. AZIN1 is frequently overexpressed and genetically altered in many cancers, including ovarian, prostate, breast, glioblastoma, and liver. Currently, Drs. Zetter and Rogers are guiding Autobahn in a target validation campaign.

The Technology Development Fund

The Technology Development Fund (TDF), established in 2009, is Boston Children's Hospital's internal mechanism for translating high-impact new technologies into the independently validated, later-stage opportunities sought by industry partners and investors. Technologies funded by TDF range from therapeutics and devices to diagnostics and vaccines in both pediatric and adult indications.

The Technology Development Fund provides:

- Mentoring and coaching through an advisory board of industry leaders in product development to identify and reach key milestones toward product development
- Funds to execute the scope of work agreed upon with the mentors
- Technical support and expertise through a network of service providers and collaborators
- Active project management to maintain focus on development goals

The 2023 Awardees:

Subcutaneous Abrasion Treatment for Stretch Marks



Arin K. Greene, MD, MMSc
Department of Plastic and Oral Surgery

Stretch marks, scars in the skin that most commonly occur when the integument is rapidly expanded (e.g., adolescence, pregnancy), affect 50% (40% of males and 70% of females) of humans. They represent a tearing of the deep layer of the skin with an intact top layer of skin. A multibillion dollar industry exists to improve the appearance of these scars using "top down" approaches with minimal efficacy and multiple treatments. These approaches go through the outside visible part of the skin and thus cause pain, erythema, bruising, and swelling requiring patients to have a prolonged recovery. The goal of these interventions is to stimulate collagen formation in the deepest layer of the skin (reticular dermis) to improve the appearance of the scars.

Dr. Greene discovered a new potential treatment method while performing liposuction to treat a patient with lipedema who also had multiple, severe stretch marks on her thighs. Using a unique instrument that also abrades the overlying skin, Dr. Greene found that after the first procedure the stretch marks where liposuction had been performed had gone away. Dr. Greene is hoping to develop a stretch mark device that "erases" stretch marks by abrading the deepest part of the skin (reticular dermis) by a subcutaneous "bottom-up" approach. Ideally, the instrument will be individually packaged for one-time use and will not require a suture closure.

Pharmacological Inhibitors of the METTL1 Oncogene



Richard Gregory, PhD

Department of Pediatric Research

Cancer is the second-leading cause of death with an estimated global cost of >\$1 trillion. One limitation in treating this devastating disease is the small number of available drug targets. Dr. Richard Gregory recently identified the tRNA methyltransferase METTL1 as a new oncogene, finding that METTL1 is recurrently amplified and/or overexpressed in various cancers. METTL1 knockdown in human cancer cellsincluding glioblastoma, liposarcoma, melanoma, and acute myeloid leukemia-leads to decreased m7G tRNA modification and stability, translational inhibition of a subset of growthpromoting mRNAs, cell cycle defects, and suppression of tumor growth in mouse xenograft models. The goal of the project is to develop pharmacological inhibitors of METTL1. The group's virtual screening of small molecules computationally docked into the active site of the METTL1 structure, and subsequent functional screening of 31 candidate inhibitors, led to the identification of a METTL1 inhibitor. Guided by medicinal chemistry consultants at HMS, Dr. Gregory's group has subsequently screened 77 commercially available related compounds in biochemical and cellular assays and found several with >5 to 10-fold increased potency and improved specificity. This structural data will help inform design sets after which chemical synthesis will be executed at highquality chemistry CROs.

Technique to Create Bone in the Upper Jaw of Children with Cleft Lip and Palate



James Maclaine, BDS
Department of Dentistry

1 in 600 children are born with cleft lip and/or palate, the most common craniofacial anomaly. A bone graft is required in pre-teen years to join the separated bones of the upper jaw. The current bone grafting technique is a major operation with a relatively high failure rate (5-20 % nationally). There are two surgical sites (the hip donor bone site and the site within the mouth) and a 6-week movement and feeding limitation post operatively.

Dr. Maclaine's group is hoping to use the TDF award to develop an alternate technique to the bone graft with a low failure rate. Unlike traditional bone grafting, this is a minor operation with one surgical site and no limitation of movement post-operatively. Additionally, the proposed technique could also replace traditional jaw (orthognathic) surgery in certain cases to harmonize the facial esthetics as well as restoring the bite. As this is their second TDF award, they are planning to further refine device design and technique, investigate route to market, develop virtual surgical planning software, and engage a consulting company to navigate the dental FDA approval process. They are hoping to bring the technology closer to the market and provide oral surgeons with an alternative to the traditional bone grafting +/- orthognathic surgery.

Novel CBD Analogs for Treatment of Pain





Clifford Woolf, MB, BCh, PhD

Jed Hubbs, PhD

Department of Neurology

Recent years have seen great inroads into discovering the genetic basis of pediatric neurologic diseases, with hundreds of single gene disorders being discovered, but treatments remain scarce. Advances in genomic technologies are beginning to point not only to critical disease genes but also methods for intervening upon them. Viral gene replacement strategies are showing promise for some conditions, but also have significant challenges.

Previously, the Yu lab successfully developed antisense oligonucleotides (ASOs) capable of boosting levels of progranulin (GRN), a genetic cause of a childhood neurodegenerative disorder (Batten disease) as well as adult onset fronto-temporal dementia (FTD), leveraging the ability of ASOs to relieve naturally occurring inefficiencies in GRN RNA splicing. A similar ASO boosting SCN1A expression in epileptic children with Dravet Syndrome is already in Phase 1/2 clinical trial

With support from Boston Children's Technology Development Program, the inventors are aiming to (1) identify at least 10 potential gene targets for ASO intervention for a variety of pediatric neurogenetic disorders, and (2) generate a genome-wide catalog of therapeutic ASO designs targeting their specific inefficiencies. By targeting existing gene loci, ASO-enhanced expression remains under the control of endogenous gene regulatory elements, allowing one to avoid potential toxicity caused by over-or misexpression.



Drug, Device, and Diagnostic Accelerator

The Drug, Device, and Diagnostic Accelerator (D3A) launched at Boston Children's Hospital in 2020. D3A is an internal accelerator focused on the development of high-potential drug, device, and diagnostic inventions that may lead to advanced commercialization and significant patient impact. Building on the successful efforts of the Technology Development Fund (TDF) and Translational Research Program (TRP), D3A works with BCH physicians and scientists to identify innovations that will benefit from expert advice, additional investment of capital, program management, and external technical resources to fast-track ideas into full-scale development.

Goals

- Enhance translation of science into novel and innovative new therapies, devices and diagnostics that meet a significant, unmet medical need for children while doing so in a capital and time-efficient manner
- · Improve commercialization and expedited clinical development awareness and skill among faculty
- Increase value of BCH scientific discoveries and expedite the translation of these into our clinical mission and/or increase ROI of research investments to BCH
- Unite similar existing programs into one comprehensive new structure that will simplify and efficiently manage high-value discovery programs

FY23 D3A Team



William Clarke, MD
Faculty Director



Mei-Mei Huang, PhD. MBA Program Manager

For more information about D3A, please email D3A@childrens.harvard.edu or contact Bill Clarke, MD, Faculty Director, at <u>William.Clarke@childrens.harvard.edu.</u>

FY23 D3A Projects

Developing a Pediatric Annuloplasty Ring



Eric Feins MD Department of Cardiac Surgery

Congenital heart disease affects nearly 1% of births in the United States, with at least 40,000 infants expected to be impacted each year. Of those infants, approximately 25% will require an invasive procedure in their first year of life. Abnormalities and dysfunction of the atrioventricular (AV) heart valves (i.e. mitral valve & tricuspid valve) are common problems across a broad range of congenital heart defects. A standard component of mitral/tricuspid valve repair is an annuloplasty, whereby the dilated valve annulus is surgically reduced back to its normal size in order to restore normal valve function and prevent future valve dilation. Unfortunately, annuloplasty rings are fixed in size and cannot be used in growing children. Doing so would restrict growth, leading to valve stenosis. Pediatric surgeons therefore must forgo annuloplasty altogether, or perform an annuloplasty with suturing techniques, which involve passing sutures through the annulus to transiently restrict its size. When compared to ring annuloplasty, suture annuloplasty is known to be less durable and can lead to inferior outcomes.

To face this unmet medical need, the D3A team and Dr. Eric Feins are developing a novel annuloplasty ring that can accommodate growth and be used for valve repair in growing children. The implant is intended to enhance the stability and durability of pediatric AV valve repair without causing growth restriction. We assembled a team with a medical device regulatory affair expert, mechanical design and testing experts, in vivo and in vitro testing CROs, and a manufacturing partner to tackle this problem. With our steady progress and new data generated, we have raised an additional \$1 million of funding from a philanthropic donation. Currently, our plan is to submit a Humanitarian Device Exemption (HDE) to the FDA in late 2024, and to initiate clinical trials in early 2025.

A novel gene therapy for Diamond-Blackfan anemia





Vijay Sankaran, MD, PhD

Richard Voit, MD, PhD Dana-Farber/Boston Children's Cancer and Blood Disorders Center

Diamond-Blackfan anemia (DBA) is a congenital red cell aplasia caused by haploinsufficiency in one of the many genes that contribute to the impaired translation of the erythroid master regulator GATA1. Current treatments for DBA are sub-optimal and are associated with considerable morbidity and healthcare expenditure. Dr. Sankaran and team proposed a novel, unified gene therapy approach, applicable to all patients with DBA. They have designed a gene therapy vector to achieve increased erythroid lineage-specific expression of GATA1 and conducted tests using in vitro DBA models and xenotransplant mouse models as well as primary bone marrow samples from DBA patients, demonstrating that this vector confers sufficient GATA1 expression to overcome the erythroid lineage commitment and maturation defects in DBA.

Based on these encouraging results, D3A joined Dr. Sankaran's team, to provide support in project management, regulatory affairs, GMP manufacture guidance and GLP tox/safety studies. With funding from the Blavatnick Therapeutic Award, D3A, DBA patient advocacy groups, philanthropic donations, and potential additional clinical trial fundings from the NIH, we expect to file an IND in late O2 2024

Issued U.S. Patents

11.576.958

Protein antigens that provide protection against pneumococcal colonization and/or disease

Malley, Richard Lu, Yingjie Zhang, Fan

11,654,149

Calmodulin inhibitors for the treatment of ribosomal disorders and ribosomapathies

Zon. Leonard Taylor, Alison

11,685,733

Compounds for treating Rac-GTPase

mediated disorder Williams, David De Vita, Serena

11.564.977

Systemic gene replacement therapy for treatment of X-linked myotubular myopathy (XLMTM)

Beggs, Alan Buj-Bello Anna Childers, Martin

11.627.949

Insertable catheter device for patch application

del Nido, Pedro

11.490.887 Suturing apparatus using autotransfer and method thereof

Liu, Kaifeng

Methods and compositions for tauopathy diagnosis and treatment

Muntel, Jan Steen, Hanno Steen, Judith

11.485.787

Agents that modulate RGMb-neogenin-BMP signaling and methods of use

DeKruyff, Rosemarie Freeman, Gordan Umetsu, Dale Yu, Sanhong

11,466,080

Methods of treating cancer using anti-LRP1 polyclonal antibodies

Watnick, Randolph

11.654.178

Cyclic prosaposin peptides and uses

thereof

Watnick, Randolph

Methods and compositions relating to exosomes

Kourembanas, Stella Mitsialis, S. Alexander Sdrimas, Konstantino

11 612 448

Medical equipment adaptable travel restraint systems

DeGrazia, Michelle

11,559,568

Short chain ceramide-based lipids and uses thereof

Chinnapen, Daniel Lencer, Wayne

11.491.480

Products and methods to isolate mitochondria

Cowan, Douglas Levitsky, Sidney McCully, James Pacak, Christina

11,583,528

FFA1 (GPR40) as a therapeutic target for neural angiogenesis diseases or disorders

Joyal, Jean-Sebastien

Smith, Lois

Seizure prediction based on comparison of biological information across wake and sleep periods

Loddenkemper, Tobias Nogueira, Adriano

11,572,543

Targeting BCL11A enhancer functional regions for fetal hemoglobin

Bauer, Daniel Orkin, Stuart Sanjana, Neville Shalem, Ophir Zhang, Feng

11,464,401

Optically guided surgical devices

Dupont, Pierre

10 730 983

Superbiocompatible polymers and hydrogels for reducing foreign body

Anderson, Daniel Langer, Robert Volkan, Yesilyurt

11.697.640

Compounds for treating proliferative diseases

Sun, Lijun Zetter, Bruce

11,713,483

Method for detection of analytes via

polymer complexes Hansen, Clinton Wong, Wesley

Immune Cells derived from induced

pluripotent stem cells

Daley, George Thuy Vo, Linda

11,560,410

Modified biotin-binding protein, fusion proteins thereof and applications

Malley, Richard Lu, Yingjie Zhang, Fan

11.685.917

Functional genomics using CRISPR-Cas systems for saturating mutagenesis of non-coding elements, compositions, methods, libraries and applications thereof

Bauer, Daniel Orkin, Stuart 11.553.687

Devices for analyzing animal behavior

Barrett, Lee Roberson, David 11.679.126

Methods to enhance microvascular engraftment of bioengineered and

primary tissues Melero-Martin, Juan

11.548.936

Compositions and methods for treating lysosomal storage diseases and disorders

Biffi Alessandra Cavalca, Eleonora

Coaptation mapping technology for heart valve assessment

Hammer, Peter Hoganson, David

Materials and methods for delivering nucleic acids to cochlear and vestibular

cells

Holt, Jeffrey Geleoc, Gwenaelle Asai, Yukako

11,464,854 Methods and compositions relating to adjuvants

Levy, Ofer Dowling, David Brazin-Lee, Helene Burkhart, David Evans, Jay Smith, Alyson

Enhanced organogenesis through manipulation of LIN28/LET-7/DIS3L2

Daley, George Osborne, Jihan Yermalovich, Alena

11 673 891

11.512.316

Imidazopyrimidine compounds and uses

thereof Levy, Ofer Dowling, David Borriello, Francesco Brightman, Spencer Feru, Frederic Scott, David

Composition comprising an antigen and a substituted imidazo[1,2-a]pyrimidine for enhancing human immune response

Levy, Ofer Dowling, David Borriello, Francesco Brightman, Spencer Feru, Frederic Scott, David

Issued U.S. Patents

11.674.140

Compositions and methods for treating facioscapulohumeral dystrophy

Kunkel, Louis Lek. Angela 11,685,782

Methods of treating cancer using LSD1 inhibitors in combination with immunotherapy

Shi. Yang Sheng. Wanqiang Sharpe, Arlene LaFleur, Martin

11,628,171

11 591 636

Method for treating brain or nerve injury

Rosenberg, Paul Benowitz, Larry

Force-controlled nanoswitch assays for single-molecule detection in complex

biological fluids Wong, Wesley Blass, Johanna Yang, Darren Hansen, Clinton

11,471,515

Restoration of tumor suppression using

MRNA-based delivery system

Zetter, Bruce Shi, Jinjun Xu, Yingjie Islam, Mohammad Farokhzad, Omid

11,559,586

Nanoparticles for treatment of choroidal neovascularization and other indications

Kohane, Daniel Wang,Yanfei

11,491,010
Optical delivery and insertion of artificial

chordae tendineae Dupont, Pierre Arnal, Gustavo Price, Karl

11,712,329 Airway stents Dupont, Pierre Kaza, Aditya Price, Karl Zhao, Zhanyue Ha, Junhyoung

11,730,769

Compositions and methods for Williams

Syndrome (WS) therapy

He, Zhigang 11.744.506

Systems and methods for analyzing

concussion biomarkers Borsook, David Moulton, Eric

Nanobody based imaging and targeting of ECM in disease and

development. Ploegh, Hidde

11,597,769

11,712,444

Compositions and methods of treating

neuronal injury Benowitz, Larry Fahrni, Christoph Morgan, Michael Rosenberg, Paul Sergeeva, Elena 11,624,093

Tumor suppressor and oncogene biomarkers predictive of anti-immune checkpoint inhibitor response

Kim, Čarla Xu, Chunxiao Wong, Kin Fillmore, Christine, Hammerman, Peter Dranoff, Glenn Akbay, Esra

11,746,154

CD1a antibodies and uses thereof

Winau, Florian

11,766,481

Glycan modified short interfering RNA

Flynn, Ryan

11,547,276

Optical bulb for surgical instrument port

Biss, David Cerier, Jeffrey MacMillin, Brian Maiorano, Anthony Masella, Benjamin

7,053,190

The Comfort Ability

Coakley, Rachael

11,517,630

Methods and compositions for regulating

gene expression Mulligan, Richard Murphy, George

11,542,328

Therapeutic and diagnostic methods

relating to cancer stem cells

Frank, Markus Frank, Natasha 11 624 054

ABCB5 positive mesenchymal stem cells as immunomodulators

Frank, Markus

Vaccine nanotechnology

Von Andrian, Ulrich Langer, Robert Zhang, LiangFang Moseman, Ashley Junt, Tobias Iannacone, Matteo Farokhzad, Omid Basto, Pamela Alexis, Frank

11,590,196

Saposin-A derived peptides and uses

thereof

Watnick, Randolph

11,510,970

Methods for treating and preventing neutrophil-derived net toxicity and thrombosis

Wagner, Denisa Thomas, Grace Martinod, Kimberly Fox, Toby De Meyer, Simon Brill, Alexander

11.484.578

Biomaterial for articular cartilage maintenance and treatment of arthritis

Murray, Martha Fleming, Braden

About the Technology & Innovation Development Office

The Technology & Innovation Development Office (TIDO) maximizes the impact of Boston Children's Hospital innovations on patient health while enhancing the research endeavor. The TIDO team is comprised of specialists in licensing, patenting, business development, marketing, startup formation and legal matters. We work closely with Boston Children's investigators and clinicians to develop innovations, protect and license intellectual property, and enable collaborations with companies at all stages of development.



About Boston Children's Hospital

Boston Children's is ranked among the best in the nation by <u>U.S. News and World Report</u>. It is home to the world's largest pediatric research enterprise, and it is the leading recipient of pediatric research funding from the National Institutes of Health. It is the primary pediatric teaching hospital for Harvard Medical School. Boston Children's treats more children with rare diseases and complex conditions than any other hospital. Its discoveries have benefited both children and adults since 1869. Today, 3,000 researchers and scientific staff, including 9 members of the National Academy of Sciences, 23 members of the National Academy of Medicine, and 12 Howard Hughes Medical Investigators comprise Boston Children's research community. Founded as a 20-bed hospital for children, Boston Children's is now a 415-bed comprehensive center for pediatric and adolescent health care. For more, visit our Discoveries blog and follow us on social media @BostonChildrens, @BCH_Innovation, Facebook and YouTube.





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