# K.I.D.S. IQ Project

## 2017 Annual Report

K.I.D.S. IQ PROJECT

Advancing drug development for
Kids with Intellectual Disability Syndromes.

**Our 2017 Annual Report details our plans to identify and accelerate research, with the goal of generating new leads and transforming lives touched by Intellectual Disability disorders. We attribute our initial success to the substantial commitment of our personal and professional community, and our network of families who struggle with Kleefstra syndrome and other Intellectual Disability disorders every day. Your generous support has allowed us to fund high-impact research that moves us closer to finding a treatment, one day at a time.**

## A Message From Our Executive Team

K.I.D.S. IQ Project began nearly three years ago, when our founders embarked on a mission to learn more about Intellectual Disability (ID) disorders and Kleefstra syndrome. After connecting with the world's leading scientists, doctors and researchers, they learned something remarkable: recent advances in technology and genetics suggest that many ID disorders, once thought to be permanent, can actually be *reversed.*

Supported by a world-class Scientific Advisory Board – including representation from SickKids, MIT, Harvard and Stanford – K.I.D.S. IQ Project has raised over $1.2 million in the last two years, funded top research labs and become the largest funder of Kleefstra syndrome drug research in the world. Perhaps most exciting of all, this past year our research project in the Netherlands identified a "hit" on a drug that showed significant promise in improving Kleefstra syndrome patient brain development.

Building on this success, we've now set our sights even higher for the year ahead, with more ambitious fundraising targets, exciting new research proposals and a vastly expanded calendar of events and resources for parents. Whether you are a sponsor, fundraiser or family member, your involvement makes you part of something incredibly special as we work to change the future for children around the world and, in the process, impact scientific discovery.

### Our Vision

Based in Canada, K.I.D.S. IQ Project is a global charitable foundation that is breaking new ground by accelerating drug discovery research to treat rare genetic disorders and reverse Intellectual Disability.

Through K.I.D.S. IQ Project's innovative model, funding is directed to high-impact, scalable research projects designed to quickly realize life-changing outcomes for more than one million children suffering from Intellectual Disability disorders.

Sincerely,

Braden Root-McCaig
Executive Director
K.I.D.S. IQ Project

Hira Verma
Board Chair
K.I.D.S. IQ Project

# Maximizing Impact

**Since founding in 2015, K.I.D.S. IQ Project has:**

- Identified the first drug in history to demonstrate effectiveness in treating Kleefstra syndrome in a lab environment, which may facilitate advances for other ID disorders;
- Built relationships and funded top research facilities around the world;
- Launched an annual conference and education platform to support families affected by Intellectual Disability disorders;
- Become the largest funder of Kleefstra syndrome research globally;
- Invested in a patient database that will be critical in future clinical trials and drug approvals.

Over
**$1.2 million**
raised to date

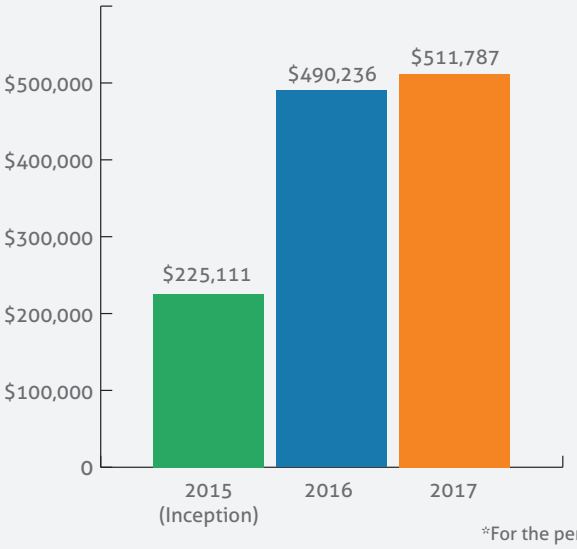
Over
**85%**
of actual and planned expenditures directed to charitable activities*

Nearly
**$800,000**
already deployed or committed to charitable activities since inception

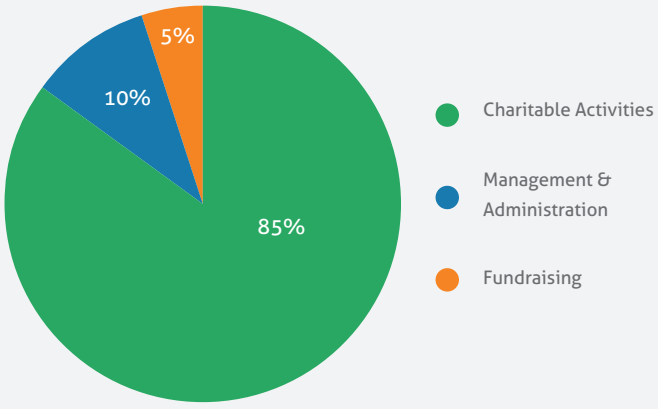
**Over 50%**
compounded annual revenue growth since inception

"When my son was diagnosed, there was nowhere to turn for information, let alone hope for the future. K.I.D.S. IQ Project helps me believe that progress is possible in Ethan's lifetime."

## Year-over-Year Revenue




## Effective Expense Management (2015-2017*)




*For the period from inception to December 31, 2017; pro forma for research expense commitments as at December 31, 2017 and further adjusted to exclude foreign exchange-related gains/losses.

# Empowering Individuals

## Founded by parents, for parents.

At K.I.D.S. IQ Project, we are completely patient focused. We understand that in order to achieve success, patients and families must be at the core of what we do. While smart science and funding are critical in finding breakthrough treatments, the active involvement of patient families and their loved ones is equally important.

Our decision to host an annual Kleefstra syndrome conference is a testament to our commitment to the Kleefstra syndrome community. By bringing researchers and families together to socialize and share the latest in treatment progress, we continue to build excitement, momentum and a mindset of cooperation and unity.

## Educational resources you can trust.

K.I.D.S. IQ Project doesn't just fund research. The foundation also provides a wide variety of educational content, including webinars, newsletters and conferences, to support parents and provide valuable information about ID disorders.

## Fighting back with patient data.

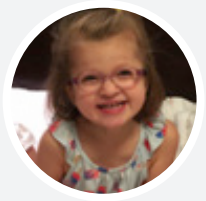
Projects currently in development at K.I.D.S. IQ Project aim to gather data directly from patients. This information is highly valuable in better understanding our patient-community and determining what treatments are most promising. If you have a child with Kleefstra syndrome and haven't already done so, please add your information to our growing patient registry online at **www.kidsIQproject.org**.

# Team Leaderboard

## Families drive progress.

Our global Walk & Roll empowers parents around the world in our search for a treatment through an opportunity to help raise funding and awareness. Beginning in 2016, this event has raised over $200,000. Thank you to the following 2017 leading teams:

**WALKING IN NATALYA'S FOOTSTEPS**



Amount Raised

**$25,000**

**NOAH'S TEAM, FOR KLEEFSTRA SYNDROME**

Amount Raised

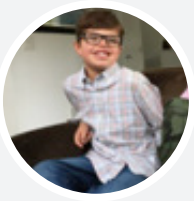
**$17,875**

**LOGAN'S WALK FOR HOPE 2017**

Amount Raised

**$16,550**

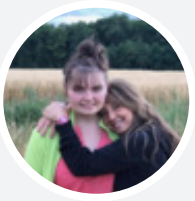
**BRENNAN'S BUDDIES**



Amount Raised

**$5,513**

**CURE FOR KLEEFSTRA 5K WALK & ROLL**



Amount Raised

**$4,575**

# From The Lab To The Clinic. Faster Than Ever.

**Whether the approach is boosting healthy proteins, repurposing existing drugs or exploring new gene therapy techniques, K.I.D.S. IQ Project is funding every innovative avenue to progress. Our high-impact, scalable research projects make it possible to identify early results, foster global collaboration and drive progress towards life-changing outcomes faster than ever before.**

## K.I.D.S. IQ Projects

### Radboud University

### (Nijmegen, The Netherlands)

**Screening a small and highly targeted library of drug compounds on human Kleefstra syndrome "patient neurons in a petri dish," with subsequent validation on a mouse model.**

**Dr. Hans van Bohkoven**
Head of Molecular Neurogentics, Radboud University Nijmegen Medical Centre

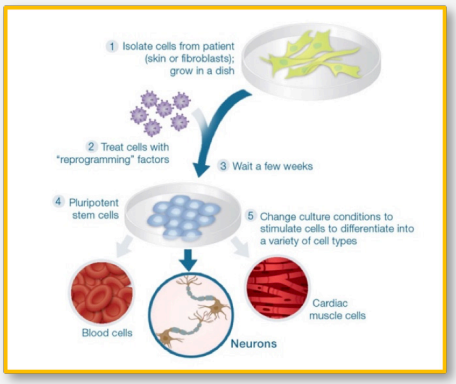
**Dr. Nael Nadif Kasri**
Assistant Professor, Radboud University Nijmegen Medical Centre

The Radboud team had previously developed Kleefstra syndrome neurons (brain cells), derived from a patient's skin sample through an innovative process involving induced pluripotent stem cell (iPSC) technology. iPSC is a relatively new technique that enables neurons to be created from patients. These neurons resided in a petri dish and were evaluated for deficiencies, as compared to neurons from a healthy patient. The team began testing the impact of selected drugs and compounds to assess their ability to improve noted deficiencies. After the initial screen of the compounds, the team identified one in particular which showed significant promise in improving neuronal deficiencies. To our knowledge, this is the first time in history that a specific drug or compound has been shown to improve or reverse Kleefstra syndrome in a lab environment, in any kind of human or animal model.

Since the discovery (i.e. identification of a drug "hit"), K.I.D.S. IQ Project began further testing and validation. This work is on-going with the research team ultimately looking to advance a drug or compound to further testing, in order to better understand behavioural and cognitive impacts. The goal of the project is to identify a potential drug candidate for testing on human Kleefstra syndrome patients in a clinical trial setting.

#### Induced Pluripotent Stem Cells (iPSC)

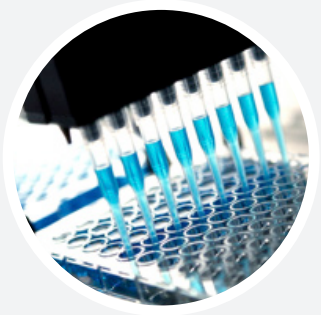
is a new technology that enables neurons (brain cells) to be derived from a patients' skin sample. This allows in vitro (lab petri dishes) analysis for discovering human neuronal phenotypes and screening drugs.




*Graphic from learn.genetics.utah.edu; text source stemcells.nih.gov*

#### High-throughput Screening (HTS)

is a method for scientific experimentation especially useful in drug discovery. Using robotics, data processing and sensitive detectors, high-throughput screening allows researchers to quickly conduct thousands of pharmacological tests and rapidly identify active drugs or compounds that modulate the drug target or phenotype.



# K.I.D.S. IQ Projects - Continued

## High-throughput Screening of Drugs to Upregulate EHMT1 (Kleefstra syndrome Gene)

**High-throughput screening (HTS) of drugs and compounds to identify those that increase the EMHT1 protein (produced by the EHMT1 gene) which is deficient or mutated in Kleefstra syndrome patients.**

Like all humans, Kleefstra syndrome patients are supposed to have two healthy copies of EHMT1. However, the gene mutations or deletions of these patients result in one copy being deficient. In identifying drugs that can successfully interact with, and stimulate increased production of EHMT1, we may be able to produce enough of the protein to compensate for the deficiency.

This project aims to leverage an HTS tool (developed and owned by K.I.D.S. IQ Project) that will be used to screen drug compounds that specifically increase the production of this important protein, in hopes of producing sufficiently high levels that compensate for the deficiency. Alongside our Scientific Advisory Board, we have identified a number of target compound and drug libraries to focus screening efforts. Once any drug "hits" are identified, extensive analysis will take place to validate the findings and a short list will be created for further testing in subsequent research experiments.

## Broad-Based Drug Screen on iPSC-derived Kleefstra Syndrome Neurons

**Multi-phase and broad-based drug screen designed to identify drugs and compounds that improve human Kleefstra syndrome patient neurons.**

Using previously developed iPSC cell lines from multiple patients, this project will subsequently convert these cells to neuronal (brain) cells for further study. These neurons will then run through multiple and complementary drug screens (up to 100,000 drugs) to maximize the potential of advancing any potential "hits."

The neurons will first be evaluated for deficiencies, as compared to neurons from a healthy patient, with the goal of identifying drugs that are able to improve noted deficiencies. This project aims to identify a potential drug candidate and advance further testing, eventually moving to human Kleefstra syndrome patients in a clinical trial setting.

## A Proven Model

HTS and iPSC technologies have been successful in advancing treatments for other diseases.

- **ALS (Lou Gehrig's Disease) and FPO (Stone Man Syndrome) are severely debilitating genetic diseases**
- **iPSC (stem cell technology) was used to identify new, already FDA-approved, drug therapies**

# Thank You To Our Supporters!

The important progress K.I.D.S. IQ Project has made would not be possible without the generosity of our supporters. We are honoured to recognize our corporate partners, whose support has allowed us to continue highly promising research towards identifying a drug treatment for Kleefstra syndrome. We are profoundly grateful for the support of our donors, without whom we would not be making unprecedented, breakthrough progress in our research.

ONEX
Scotiabank
STIKEMAN ELLIOTT
FRIED FRANK
BMO Capital Markets
KPMG
EY Building a better working world
MEDCAN LIVE WELL, FOR LIFE.
Blakes LAWYERS
ATS
FIRST ASSET EXCHANGE TRADED FUNDS
GOODMANS LLP
KORN FERRY Executive Search | HayGroup | Futurestep
CIBC 150
RBC Capital Markets
Goldman Sachs
NATIONAL BANK FINANCIAL MARKETS
HSBC
EgonZehnder
Torkin Manes Barristers & Solicitors
Morgan Stanley
UBS
xpv water partners
ALTASI PARTNERS
CINEPLEX
pwc
OSLER
LINAMAR Power to Perform
OMERS
Deloitte.

> There are drugs currently in development that have the potential to reverse Intellectual Disability disorders, and dramatically improve the lives of children and families around the world. I encourage you to support K.I.D.S. IQ Project and their important work of making these life-changing treatments a reality.

**Brian Porter, President & CEO**
Scotiabank

# Team

## OUR TEAM - Experienced and specialized, with a proven track record:

### Board Chair – Hira Verma
Investment banking veteran, advising on over $14 billion of transactions including mergers, acquisition and financings

### Board Member – Andy Klump
Successful clean energy entrepreneur and international business professional

### Board Member – Samer Abughaman
Private equity expert, specializing in social impact and healthcare

### Executive Director – Braden Root-McCaig
Social entrepreneur and former political strategist

### Scientific Director – Paul Smith
Pharma expert responsible for bringing 12+ research drugs to clinical trials (incl. first FDA-approved for Multiple Sclerosis)

### Marketing Director – Will Connor
Brand and digital design expert with over 15 years' of global client experience

### Co-Chair, Parent Leadership Council – Neha Verma
10+ years in investment, strategic advisory and capital raising roles; currently a Principal at Onex

### Co-Chair, Parent Leadership Council – Lindsay Klump
Stakeholder relations expert, recently overseeing $61 million in sponsorship at the Shanghai Expo

## OUR SCIENTIFIC ADVISORY BOARD

K.I.D.S. IQ Project is guided by individuals from industry-leading institutions, including SickKids, MIT, Harvard and Stanford. By coordinating research efforts and insights from across the globe, we can turn actionable hypotheses into life-changing outcomes.

### Harvey Lodish
- MIT Professor and Board of Trustees of Boston Children's Hospital
- Trained two Nobel Laureates
- Track record of curing disease (Gaucher Disease)

### Ronald Cohn
- Chair of the Department of Paediatrics at the University of Toronto and Paediatrician in Chief of the Department of Paediatrics at the Hospital for Sick Children, Toronto
- Previously at Johns Hopkins

### Sunny Kumar
- Advisor to healthcare companies on technology, commercialization and investment

### James Kramer
- Lab at University of Western Ontario covering Intellectual Disability disorders

### Brian Van Ness
- Professor and former Head of the Department of Genetics, Cell Biology and Development at University of Minnesota

### Neil Kumar
- CEO of BridgeBio, a company specializing in rare genetic disorders
- Formerly a Principal at Third Rock Ventures

Massachusetts Institute of Technology · bridgebio · SickKids · Western University · Canada · Stanford University · University of Minnesota

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**K.I.D.S. IQ Project** is a "doing business as" name for **International KS Foundation**, a Canadian registered charity with BN/Registration Number 820787992RR0001.