Who We Are

Owned & Operated by

Mount Sinai Hospital
Sinai Health System
Joseph & Wolf Lebovic Health Complex

SickKids®

Facility Partner for Infection & Inflammation Core

McGill University Research Centre on Complex Traits

Operations & Maintenance Funding Partners

INNOVATION.CA
Canada Foundation for Innovation

GenomeCanada

Ontario Genomics

The Team

<table>
<thead>
<tr>
<th>Number of full-time equivalent (FTE)*</th>
<th>2017-2018</th>
</tr>
</thead>
<tbody>
<tr>
<td>Administrative personnel</td>
<td>18.2</td>
</tr>
<tr>
<td>Technical and scientific personnel</td>
<td>97.3</td>
</tr>
</tbody>
</table>
By The Numbers

The Budget

Annual Operating Expense
$14,184,125

Our Customers

Type and Distribution of 971 Users (F2018)

- Ontario: 85%
- USA: 7%
- Rest of Canada: 4%
- International: 4%

Annual Revenue
$14,184,125

- User Fees: 62%
- CFI MSI: 21%
- Institutions: 8%
- Federal Gov't (non-CFI): 7%
- Provincial Gov'ts: <1%
- Corporations: 2%
What We Do

- Infection & Inflammation
- Pathology
- Clinical Phenotyping
- Imaging
- Cryopreservation & Recovery
- Model Production
- Accelerating discoveries. From nose to tail.
- Animal Holding
The Impact We Can Make

July to December 2016
- TCP provides services and data to support Fabry disease project

January 2017
- Manuscript submitted for publication

In Vivo Toxicology Study of Our Therapeutic Product: LV/AGA-Transduced Fabry Patient CD34+ Hematopoietic Cells

Ju Huang,1 Aneal Khan,2 Bryan Wachter,3 Michel Boutin,7 Michael Rothe,8 Axel Schambach,8,10 Armand K. Rassoulzadegan,9 C. Anthony Rupar,9 Christiane Eiche,11,12 and Oliver G. Fiebich6

1University Health Network, Toronto, ON, Canada; 2Arcturus Therapeutics, San Diego, CA, USA; 3Department of Medicine, University of Calgary, Calgary, AB, Canada; 4Department of Medical Biophysics, University of Toronto, Toronto, ON, Canada; 5Department of Medical Biophysics, University of Western Ontario, London, ON, Canada; 6Department of Molecular and Medical Pharmacology, David Geffen School of Medicine, University of California, Los Angeles, CA, USA; 7Department of Pathology, University of Calgary, Calgary, AB, Canada; 8Department of Medical Biophysics, University of Toronto, Toronto, ON, Canada; 9Department of Medical Biophysics, University of Western Ontario, London, ON, Canada; 10Department of Microbiology and Immunology, University of Alberta, Edmonton, AB, Canada; 11Department of Medical Biophysics, University of Western Ontario, London, ON, Canada; 12Department of Medical Biophysics, University of Toronto, Toronto, ON, Canada

To investigate the potential in vivo toxicity of the LV/AGA-transduced Fabry patient CD34+ hematopoietic cell product, we used our NSF mouse model for an additional xenograft study. To do this under optimal conditions, the NSF mouse line was re-derived at the Toronto Centre for Phenogenomics (TCP) behind a barrier, and a cohort of 7-week-old mixed-gender mice was selected for these experiments. CD34+ hematopoietic cells isolated from a Fabry patient (no. 15-220) were transduced overnight with vehicle (mock) or LV/AGA at an MOI of 10, and 1 × 10^6 cells were infused into recipient mice 1 day after they were irradiated and treated with antibody against mouse CD122.15 Half of the mice were killed on day 7, and the remainder were killed on day 28. Mouse weight, body and dermal condition, general appearance, behavior, metabolic parameters, and complete blood counts were assessed.
Dear TCP:
Our clinical trial submission for Fabry disease was recently approved by Health Canada! The mouse toxicology study done by your facility was included in our CTA application. Thank you again for all your good work!
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April to May 2017
- Feedback from the customer

October 2017
- Results of Fabry disease Phase I clinical trial released

Press Releases

AVROBIO Reports Six-Month Data Showing First Patient Treated for Fabry Disease Achieved Normal Plasma Enzyme Activity with AVR-RD-01 Gene Therapy

First patient with Fabry disease who received a single dose of lentiviral gene therapy achieved normal plasma α-galactosidase A enzyme activity within 45 days and has maintained those levels for six months. Data presented at 59th Annual Meeting of Japanese Society for Inherited Metabolic Diseases.

Cambridge, MA, October 12, 2017 – AVROBIO, Inc., a clinical-stage biotechnology company developing transformative, life-changing gene therapies for rare diseases, today announced initial six-month clinical data from the first patient treated with AVR-RD-01, its lentiviral gene
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June 2018
• Initial Public Offering

Press Releases

AVROBIO, Inc. Announces Pricing of Initial Public Offering

CAMBRIDGE, Mass., June 20, 2018 (GLOBE NEWSWIRE) -- AVROBIO, Inc. (the "Company"), a Phase 2 clinical stage gene therapy company focused on developing potentially curative ex vivo lentiviral-based gene therapies to treat rare diseases following a single dose, today announced the pricing of its initial public offering of 5,247,958 shares of common stock at a public offering price of $19.00 per share, before