Established in 2016
Developing transformative therapies for rare, often fatal, diseases
Advancing two development programs with near-term milestones:
- Potential 2019 BLA approval
- Potential registrational study in 2020
Operations in Cambridge, Durham and Basel
Part of the Roivant family
Leadership Team

Deep Experience in Drug Development, Commercialization and Rare Disease

Rachelle Jacques  
CEO

Alan Kimura, MD, PhD  
Chief Medical Officer

George Elston  
CFO

Morgan Molloy  
Chief Commercial Officer

Alex Tracy, PhD  
VP, Manufacturing

PROPRIETARY – FOR INTENDED AUDIENCE ONLY – NOT FOR REPRODUCTION
Platform Technologies

Building a high-value pipeline focused on two well-characterized platform technologies:

- **RVT-802 BLA ACCEPTED**
- **T CELL GENERATION** for immuno-deficiencies (lead asset RVT-802)
- **RECOMBINANT HUMAN ACID CERAMIDASE** for excess ceramide buildup (lead asset RVT-801)
Our Formula for Rapid Therapeutic Development & Commercialization

Science + Partnership = Speed

Identify well-characterized scientific platform(s) with multi-indication potential

Establish strong collaborations with originating partners

Rapidly test and validate science in indication areas of high unmet need

Extend Value, Access

Enhance capacity for development in follow-on indications

Drive adoption and access via creative value-based models
<table>
<thead>
<tr>
<th>Platforms</th>
<th>Compound and Status</th>
<th>Expected Registration</th>
</tr>
</thead>
</table>
| T CELL GENERATION | Lead Asset: RVT-802  
Pediatric Congenital Athymia  
• BLA accepted June 2019  
• Potential approval in December 2019 | 2019 |
| RECOMBINANT HUMAN ACID CERAMIDASE  
for harmful ceramide buildup | Lead Asset: RVT-801  
Acid ceramidase deficiency  
presenting as Farber disease  
• Single study (ADVANCE) to commence in 2020 | 2022 |

Investigational drugs. Have not been approved for any indication and subject to health authority approval.
Pediatric Congenital Athymia: A Rare, Deadly Immunodeficiency

Untreated Children with Congenital Athymia Typically Die Before the Age of 2

Survival of Untreated Patients (n=49)

Survival Rate

0% 20% 40% 60% 80% 100%

1 3 5 7 9 11 13 15 17 19 21 23 25 27 29 31 33 35

Months

6% at 24 months

Sources: Data on File

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RVT-802: A Novel One-Time Therapy for Pediatric Congenital Athymia

Background

• Built on research and development collaboration with Duke University
• Derived from infant thymus tissue removed during pediatric heart surgery
  • Surgeon typically removes about ¼ of infant’s thymus (which would otherwise be discarded)
• Developed with proprietary manufacturing, clinical know-how
  • Thymus tissue is aseptically processed and cultured
  • Donor thymocytes depleted during process
  • Multiple modifications transform donor thymus tissue into a regenerative medicine
**How it works**

- RVT-802 is implanted into patient quadriceps muscles
- Patient bone marrow stem cells migrate to RVT-802 and develop into naïve T cells that are immunocompetent
  - Thymopoiesis in biopsies occurs within 2-3 months of implantation
  - Naïve T cells (indicative of restored thymic function) detected in peripheral blood within ~6 months
  - *T cells are tolerant of both donor and recipient*
RVT-802 Survival: Compelling Case Supported by Long-Term Data

- In 85 RVT-802 treated patients with congenital athymia, Kaplan-Meier estimated survival rates at Year 1 and Year 2 were 76% and 75%, respectively.
- For patients surviving 12 months post-treatment, there was a 93% probability of surviving 10 years post-treatment.

Sources: Data on File
RVT-802 Ambition: Provide Every Congenital Athymia Patient Access to an Approved One-Time Therapy

For each patient with congenital athymia treated under INDs, on average 3 or more were not able to be reached.

Sources: Data on File
### RVT-802’s Potential Value for Patients Signaled through Multiple Regulatory Agency Designations

<table>
<thead>
<tr>
<th>Regulatory Agency</th>
<th>Designations Granted</th>
</tr>
</thead>
</table>
| **FDA**           | ✓ Regenerative Medicine Advanced Therapy (RMAT)  
|                   | ✓ Breakthrough Therapy  
|                   | ✓ Rare Pediatric Disease  
|                   | ✓ Orphan Drug |
| **EMA**           | ✓ Advanced Therapy Medicinal Product (ATMP)  
|                   | ✓ Orphan Drug |

RVT-802: Potential to become the first FDA-approved RMAT-designated therapy

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Maximizing Patient Access by Advancing a Value-Based Approach

Value-Based Access Approach to RVT-802
Farber Disease: A Devastating Lysosomal Storage Disease

**ASAH1** gene mutations (autosomal recessive) → Acid ceramidase enzyme deficiency (lysosomal) → Ceramide accumulation → Macrophage-driven inflammation (local and systemic)

Of 42 patients enrolled in our Farber Natural History study, 20 of the 22 deceased were under the age of 6.
RVT-801: Recombinant Human Acid Ceramidase (rhAC)

Being developed as an enzyme replacement therapy (ERT) for acid ceramidase deficiency manifesting as Farber disease

<table>
<thead>
<tr>
<th>Natural History Study</th>
<th>Defined Regulatory Pathway</th>
<th>Global Diagnostic Pathway</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Natural history study underway to strengthen disease understanding</td>
<td>• ERT is an established treatment pathway</td>
<td>• Pioneering a genetic test to improve patient ID, uncover misdiagnoses</td>
</tr>
<tr>
<td>• Strong publication cadence planned over next 12 months, including first comprehensive overview of all Farber-associated variants</td>
<td>• Clinical trial in Farber disease planned to commence 2020</td>
<td>• Out of 66 genetic mutations now identified, only 9 are in ClinVar</td>
</tr>
<tr>
<td>• 8 sites across 7 countries participating in study</td>
<td>• 8 sites across 7 countries participating in study</td>
<td>• Primary endpoint: reduction in nodule count</td>
</tr>
</tbody>
</table>
Potential Future Value Inflection Points

Expected Near-Term Milestones

- Implementation of a novel, value-based access model for RVT-802
- Interim data from RVT-801 natural history study to support and inform diagnosis and registrational study

Ongoing Value Creation

- RVT-802 BLA and approval, with potential to be the first FDA approved therapy with RMAT designation
- Expansion into new T cell generating indications
- Expansion to address CNS involvement in Farber and SMA-PME
- In-license or partnership for additional pipeline assets

Initiation of single registrational study for RVT-801 in Farber disease