Agreement to acquire shares of Orchard Therapeutics
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Agenda

Agreement to acquire shares of Orchard Therapeutics
Director of the Board, Senior Managing Executive officer and Chief Medical Officer  Takeyoshi Yamashita, Ph.D.

Q&A
Director of the Board, Senior Managing Executive officer and Chief Medical Officer  Takeyoshi Yamashita, Ph.D.
Managing Executive Officer, Head of Finance  Motohiko Kawaguchi
## Transaction Summary

<table>
<thead>
<tr>
<th>Items</th>
<th>Summary</th>
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</thead>
</table>
| **Target**                   |   ◼ Orchard Therapeutics plc (London)  
|                              |     — Listed on NASDAQ                                                                                                                                 |
| **Purchase Price**           |   ◼ $16.00 per ADS / approx. $387.4 million (approx. JPY 57.3 billion)  
|                              |     — Orchard shareholders will hold additional contingent value rights (CVR) of $1.00 per ADS.  
|                              |     — Additional $1.00 CVR will be paid for a total of $17.00 per ADS, or approximately $477.6 million (approx. JPY 70.7 billion) if OTL-200 is approved by the U.S. Food and Drug Administration for the commercial marketing and sale in the U.S. |
| **Funding Method**           |   ◼ Cash on balance sheet                                                                                                           |
| **Financial Impact**         |   ◼ To be announced once allocation of goodwill and intangible assets are determined                                                |
| **Transaction Structure and Process** | ◼ Scheme of Arrangement (SoA)  
|                              |     — Requires the approval by Orchard’s shareholder meeting, UK court, and regulatory authorities  
|                              |     — Closing is expected in 2024Q1 through implementation of SoA                                                                  |

*Refers to the amount required to make payments related to all outstanding Orchard common shares, ADS purchases, options, Restricted Stock Units, and other instruments.
Strategic Rationale of The Transaction

Our purpose of the acquisition:

- To enrich our pipeline to address the UMNs* for which there is still no cure
- To obtain capabilities in Cell Gene Therapy R&D to address the future UMNs

* Unmet medical needs

Our New Vision toward 2030

Kyowa Kirin will realize the successful creation and delivery of life-changing value* that ultimately makes people smile, as a Japan-based Global Specialty Pharmaceutical company built on the diverse team of experts with shared passion for innovation.

- Strengthen the business following Crysivia and Poteligeo as a Global Specialty Pharmaceutical company
- Enhance ability to address UMNs in the future by combining with our strength in biologics
- Commitment to life by providing not only pharmaceuticals but also treatments

* Make patient smile through dramatic improvement in quality of life by identifying the unmet medical needs of people battling with medical conditions and by creating and supplying new drugs or services that help them overcome those challenges
# Overview of Orchard Therapeutics

## Orchard Therapeutics plc

<table>
<thead>
<tr>
<th>Locations</th>
<th>United Kingdom (HQ), United Sates, Netherlands, France, Italy, Germany, Switzerland, Sweden</th>
</tr>
</thead>
<tbody>
<tr>
<td>Business</td>
<td>Biopharmaceutical company with specialty in Hematopoietic Stem Cell Gene Therapy (HSC-GT)</td>
</tr>
<tr>
<td>Employees</td>
<td>166 employees (as of Dec. 2022)</td>
</tr>
</tbody>
</table>

## Product & Clinical Pipelines

<table>
<thead>
<tr>
<th>Clinical Asset</th>
<th>Target disease</th>
<th>Status</th>
<th>Next Catalyst</th>
<th>Expected timing of Catalyst</th>
</tr>
</thead>
<tbody>
<tr>
<td>OTL-200 (Libmeldy®)</td>
<td>MLD</td>
<td>Launched in Europe File in US</td>
<td>FDA approval</td>
<td>PDUFA: March 18, 2024</td>
</tr>
<tr>
<td>OTL-203</td>
<td>MPS-IH</td>
<td>Ph1/2</td>
<td>Initiating pivotal study</td>
<td>Q4 2023</td>
</tr>
<tr>
<td>OTL-201</td>
<td>MPS-IIIA</td>
<td>Ph1/2</td>
<td>PoC data readout</td>
<td>Q1 2024</td>
</tr>
</tbody>
</table>
What is Hematopoietic Stem Cell Gene Therapy (HSC-GT)?

HSC-GT allows delivery of gene-modified blood/immune cells to any part of the body.

What is Hematopoietic Stem Cell Gene Therapy (HSC-GT)?

**Treatment Process**

1. Cells are collected from the patient
2. Blood stem cells are selected and purified
3. A working copy of the gene is inserted into the cells using a viral vector. Gene-corrected cells are frozen, or cryopreserved
4. The patient is conditioned to receive treatment
5. The gene-corrected cells are infused into the patient

**Durable Efficacy**

- Stable viral integration through multiple cell divisions
- Potential for “One time treatment in life”
Libmeldy® (OTL-200, atidarsagene autotemcel)

- MLD (Metachromatic Leukodystrophy)
  - Fatal genetic CNS disorder
  - Rapid and irreversible loss of motor and cognitive function
  - In its most severe form, most children pass away within five years of symptom onset

Severe Motor Impairment Free Survival (sMFS)
Interval from birth to first occurrence GMFC-MLD ≥ 5 (no locomotion and unable to sit) or death

Treatment with OTL-200 resulted in statistically significant and clinically meaningful improvement in sMFS in the PSLI (p<0.001), PSEJ (p=0.042) and ESEJ (p<0.001) MLD subgroups compared to disease natural history.

Potential annual market opportunity for Libmeldy® across all patient segments assuming an average per patient net price of $2.5M and universal newborn screening

Ref.) Orchard Therapeutics plc, Q2 2023 Financial Results and Webcast https://ir.orchard-tx.com/static-files/9fed8b65-2fd9-491a-97c0-69bf6595c0c3

2. The sale price of Libmeldy® will vary from jurisdiction to jurisdiction and could vary for a variety of reasons, some of which are outside of the company’s control. The net price utilized on this slide is for illustrative purposes only and is not an estimate or prediction of the average net price of Libmeldy® globally.
Development Pipelines: OTL-203, OTL-201

OTL-203

- **Indication:** MPS-IH\(^1\)
  - Multisystemic neurometabolic condition affecting cognition, growth and skeletal function
  - \(\sim 1:100,000\) live births; NBS\(^2\) established in some geographies, incl. U.S.

- **Current Status**
  - Interim PoC results published in NEJM\(^3\)
  - Moving into a Pivotal (Ph3) study in 2H 2023

OTL-201

- **Indication:** MPS-IIIA\(^4\)
  - Accumulation of substrate heparan sulfate leading to severe CNS degeneration w/ somatic manifestations
  - Development slows from 3 years of age, followed by cognitive decline, behavioral disturbances, loss of skills and eventual death, No successful treatment options

- **Current Status**
  - PoC study (Ph1/2) is ongoing
  - Biochemical/clinical data reported in 2023 ASGCT meeting

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Expected Synergy

Further Development as a Global Specialty Pharmaceutical Company

- To be a Japan-based Global Specialty Pharmaceutical Company providing life-changing value to high unmet medical needs in concert with our existing business through Crysvita and Poteligeo

Reinforcement of New Drug Discovery & Development

**Kyowa Kirin**
- Experience and expertise of R&D and commercialization in Biologics and Antibody Drugs
- Proprietary next-generation antibody technology
- Continuous efforts to new modalities

**Orchard Therapeutics**
- Experience and know-how in the marketing HSC-GT1 in EU
- High technology in HSC-GT1 (Research, CMC, SCM, etc.)
- Patient access and collaboration with medical institutions
- Efforts and experience to new modalities

- A key step toward delivering advanced value to patients
  - Pursuing the potential for “One-time treatment in life”
  - Challenge to correct the underlying cause of a genetic disease
  - Personalized medicine / Precision medicine
  - Providing treatment beyond the existing drugs

- Address a broader range of UMNs

1. Hematopoietic Stem Cell Gene Therapy
Conclusion

The acquisition of Orchard Therapeutics enables us to:
- Enrich pipelines to address the UMNs for which there is still no cure
- Gain capabilities in HSC-GT R&D to address the future UMNs

- Delivery of Gene-Corrected Cells to Multiple Organ Systems
- Durable efficacy, potential for “One Treatment in Life”

It will be synergistic with the businesses of Crysvita and Poteligeo, contributing to further development as a Global Specialty Pharmaceutical company.

Moreover, combining Kyowa Kirin’s technology and experiences, represented by biologics, and Orchard’s expertise in advanced HSC-GT will significantly enhance our drug discovery and development capability in the long term.

Kyowa Kirin will realize the successful creation and delivery of Life-changing value that ultimately makes people smile.
Strategic Investment ～For successful creation and delivery of life-changing value

- Licensing-in and M&A investments to strengthen the portfolio
  - Development pipeline with synergies with Crysvita and Poteligeo
    - Bone, Mineral
    - Hematologic oncology
  - Implementing the strengths of each region
    - Nephrology
    - Hematology / Oncology
    - Immunology

- Investment in science and technology to create new strengths
  - Investments aimed at acquiring new drug discovery technologies and early pipelines and accelerating cooperation and collaborations
  - VC investment and CVC activities for exploring and accessing information.

2021-2025 Cash Allocation*

- Cash to be newly generated during the FY2021-2025 MTBP (cumulative)
- Operating cash flow before deduction of R&D expenses: ¥800 billion or higher (Operating CF + R&D expenses)
- Cash on hand: Approximately ¥300 billion (at the end of 2020)
- + Borrowing capacity