Corporate Overview

At the Forefront of Therapies for Rare Diseases

June 2022
Forward-Looking Statements

This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product or to treatment sites. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, U.K., Japan, the U.S. and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, commercialization and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. We refer you to the Annual Report on Form 10-K for the year ended December 31, 2021, and on Form 10-Q for the quarter ended March 31, 2022. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

Non-GAAP Financial Measures

In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.
A Rare Company

Patient-Dedicated, Rare Disease Biotechnology Company with Sustained Double-Digit Revenue Growth, a Global Commercial Infrastructure, and Late-stage Development Capabilities

Gene Therapy PLATFORM
Leveraging Experience in Protein Engineering & Glycobiology

EMPLOYEES in 27 Countries

AT-GAA
a Two-Component Therapy Under Global Regulatory Reviews for Pompe Disease

$350M-$365M
FY22 Global Galafold Revenue at CER

GLOBAL COMMERCIAL ORGANIZATION

GALAFOLD & AT-GAA
Cumulative $2B Peak Potential

$411M Cash as of 3/31/22

PROFITABILITY expected in 2023
2022 Strategic Priorities to Drive Value

1. Double-digit Galafold growth (15-20%) with revenue of $350M to $365M at CER¹

2. Secure FDA approval and positive CHMP opinion for AT-GAA

3. Initiate successful, rapid launch in U.S. for AT-GAA

4. Advance best-in-class next-generation genetic medicines and capabilities

5. Maintain strong financial position on path to profitability

¹2022 Galafold revenue guidance is based on the average exchange rates for 2021
### Amicus Pipeline

Streamlined Rare Disease Pipeline with Focus on Fabry Disease and Pompe Disease

<table>
<thead>
<tr>
<th>INDICATION</th>
<th>DISCOVERY</th>
<th>PRECLINICAL</th>
<th>PHASE 1/2</th>
<th>PHASE 3</th>
<th>REGULATORY</th>
<th>COMMERCIAL</th>
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</thead>
<tbody>
<tr>
<td><strong>FABRY FRANCHISE</strong></td>
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<tr>
<td>Galafold® (migalastat)</td>
<td>ODD</td>
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<tr>
<td>Fabry Gene Therapy</td>
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<tr>
<td><strong>POMPE FRANCHISE</strong></td>
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<tr>
<td>AT-GAA (ciglucosidase alfa + miglustat)</td>
<td>BTD ODD</td>
<td></td>
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<tr>
<td>Pompe Gene Therapy</td>
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<tr>
<td><strong>OTHER</strong></td>
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<tr>
<td>CLN3 Batten Disease Gene Therapy</td>
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<tr>
<td>Next-Generation Research Programs</td>
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**Legend:**
- **ODD** - Orphan Drug Designation
- **BTD** - Breakthrough Therapy Designation
Positioned for Significant Value Growth

Focused on Execution and Driving Sustainable Double-Digit Revenue Growth on Path to Profitability

- Continue to bring Galafold® to as many patients as possible, sustain double-digit revenue growth
- Successful launch of AT-GAA for people living with Pompe disease
- Advance next-generation gene therapies in Fabry and Pompe diseases
- Fully leverage global capabilities and infrastructure as a leader in rare diseases
- Achieve self-sustainability and profitability in 2023
Galafold® (migalastat) Continued Growth

... building a leadership position in the treatment of Fabry disease
Global Fabry Market

Global Fabry Disease Market Growth Continues to be Driven by Diagnosing New Patients in Addition to the Introduction of Galafold

- Fabry Disease is believed to be significantly underdiagnosed
  - Newborn screening studies suggest Fabry could be one of the more prevalent human genetic diseases (~1:1,000 to ~1:4,000 incidence)
- In 2021, Galafold was the fastest growing medicine for Fabry disease and the greatest contributor to Fabry market growth
  - Introduction of Galafold has led to market expansion with 800+ naive patients diagnosed and treated for the first time

$0$ $500$ $1,000$ $1,500$ $2,000$ $2,500$ $3,000$


Global Fabry market to exceed $1.9B in 2021 and tracking toward ~$2.6B by 2025

1Global market measured by reported sales of approved therapies for Fabry disease – 2025 sales projected using 8% CAGR
Galafold Success (as of March 31, 2022)

Building on Galafold’s Success and Leveraging Leadership Position to Drive Continued Growth

Galafold is first and only approved oral treatment option with a unique mechanism of action for Fabry patients with amenable variants.

- 1,384 Amenable Mutations Included in EU Label
- $78.7M +18.5% YoY 1Q22 Galafold Revenue
- 2022 Galafold Revenue Guidance at CER $350M-$365M
- 40+ Countries with Regulatory Approvals
- 350 Amenable Variants in U.S. Label
- Continued Geographic Expansion in 2022

Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit [https://www.amicusrx.com/pi/Galafold.pdf](https://www.amicusrx.com/pi/Galafold.pdf). For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).
Galafold Quarterly Trends

Q1 Reported Revenue Growth of +18.5% to $79M - Operational Growth of +23.5% at CER

- Global mix of switch (~55%) and previously untreated patients (~45%)
- Compliance and adherence over 90%+
- Continue to support diagnostic initiatives to drive a shorter pathway to diagnosis
- Expect non-linear quarterly growth to continue due to uneven ordering patterns
- Distribution of Galafold sales by quarter in past 3 years:

<table>
<thead>
<tr>
<th>Quarter</th>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
<th>Q4</th>
</tr>
</thead>
<tbody>
<tr>
<td>3 Year Avg.</td>
<td>21%</td>
<td>25%</td>
<td>26%</td>
<td>28%</td>
</tr>
</tbody>
</table>
Galafold Growth Opportunity

$1B Annual Sales Opportunity at Peak

**Sustained double-digit revenue growth:**
- 1Q operational revenue growth of +24%

**Near-term growth to $500M driven by:**
- Continued penetration into existing markets
- Expansion into new geographies
- Broadening of labels

**Long-term growth towards peak sales potential driven by:**
- Penetration of the diagnosed untreated population
- Increase in newborn screening and diagnostic initiatives
- Strong intellectual property rights, including COM protection through 2038

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**Sustained double-digit revenue growth:**
- 1Q operational revenue growth of +24%
Galafold Initiatives

Building the Body of the Evidence around Galafold

- Broadening Labels: Adolescents and Additional Variants
- Publications and Medical Presentations
- Over 500 Patients Enrolled in a Global Registry
- Ongoing and Planned Phase IV Studies
- Strengthening our IP Portfolio
AT-GAA
(cipaglucosidase alfa + miglustat)

... potential to establish a new standard of care for people living with Pompe disease
Pompe Disease Overview

Pompe is a Severe and Fatal Neuromuscular Disease Caused by the Deficiency of Lysosomal Enzyme GAA

Estimated incidence of ~1:28,000; newborn screening suggests significant underdiagnosis

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

Age of onset ranges from infancy to adulthood

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

Majority of patients on current standard of care decline after ~2 years

~$1.2B+ global Pompe ERT sales

Respiratory and cardiac failure are leading causes of morbidity and mortality

1. Based on 12 months ended December 31, 2021. Source: Sanofi Press Release
AT-GAA: An Innovative Approach to Pompe Disease

- AT-GAA is a two-component therapy combining ATB200, an ERT, with AT2221, an orally administered enzyme stabilizer.
- Consists of a naturally occurring cell line that can be properly processed within the lysosome to its mature form which is required to optimally break down glycogen\(^1\).

\(^1\) Selvan et al. 2021, J Biol Chem 2021 Jan-Jun;296:100769

ERT: Enzyme Replacement Therapy
### Phase 3 PROPEL Study Results

Primary, Key Secondary and Biomarker Endpoint Heat Map

**Endpoints Across Motor Function, Pulmonary Function, Muscle Strength, PROs, and Biomarkers Favored AT-GAA over alglucosidase alfa**

<table>
<thead>
<tr>
<th>Endpoints</th>
<th>Overall population</th>
<th>ERT-experienced</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Cipaglucosidase alfa/miglumstat n=85</td>
<td>Alglucosidase alfa/placebo n=37</td>
</tr>
<tr>
<td></td>
<td>Baseline, mean CFBL at week S2, mean (SE)</td>
<td>Baseline, mean CFBL at week S2, mean (SE)</td>
</tr>
<tr>
<td><strong>Motor function</strong></td>
<td></td>
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<tr>
<td>6MWD, m</td>
<td>357.9 ± 20.8 (4.6)</td>
<td>351.0 ± 7.2 (6.6)</td>
</tr>
<tr>
<td>GSGC total score</td>
<td>14.5 ± -0.5 (0.3)</td>
<td>14.5 ± 0.8 (0.3)</td>
</tr>
<tr>
<td>10-meter walk, s</td>
<td>9.7 ± -0.5 (0.6)</td>
<td>9.6 ± 1.9 (1.0)</td>
</tr>
<tr>
<td>4-stair climb, s</td>
<td>14.1 ± -8.5 (7.9)</td>
<td>8.2 ± 0.3 (1.0)</td>
</tr>
<tr>
<td>Gower's maneuver, s</td>
<td>10.8 ± -0.3 (0.7)</td>
<td>19.8 ± -2.2 (1.4)</td>
</tr>
<tr>
<td>Rising from chair, s</td>
<td>13.6 ± -10.2 (9.7)</td>
<td>4.5 ± -0.5 (0.7)</td>
</tr>
<tr>
<td><strong>Pulmonary function</strong></td>
<td></td>
<td></td>
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<tr>
<td>FVC, % predicted</td>
<td>70.7 ± -0.9 (0.7)</td>
<td>69.7 ± -4.0 (0.8)</td>
</tr>
<tr>
<td>MIP, % predicted</td>
<td>61.8 ± 2.1 (2.1)</td>
<td>59.9 ± -2.7 (2.8)</td>
</tr>
<tr>
<td>MEP, % predicted</td>
<td>70.7 ± 0.6 (2.4)</td>
<td>65.1 ± -1.6 (2.1)</td>
</tr>
<tr>
<td><strong>Muscle strength</strong></td>
<td></td>
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<tr>
<td>Lower MMT score</td>
<td>28.0 ± 1.6 (0.4)</td>
<td>27.7 ± 0.9 (0.4)</td>
</tr>
<tr>
<td>Upper MMT score</td>
<td>34.3 ± 1.5 (0.4)</td>
<td>34.7 ± 0.7 (0.6)</td>
</tr>
<tr>
<td>Total MMT score</td>
<td>62.3 ± 3.1 (0.7)</td>
<td>62.4 ± 1.4 (0.8)</td>
</tr>
<tr>
<td><strong>PROs</strong></td>
<td></td>
<td></td>
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<tr>
<td>PROMIS®-Physical Function</td>
<td>66.9 ± 1.9 (0.8)</td>
<td>68.0 ± 0.2 (1.8)</td>
</tr>
<tr>
<td>PROMIS®-Fatigue</td>
<td>22.3 ± -2.0 (0.6)</td>
<td>21.1 ± -1.7 (1.1)</td>
</tr>
<tr>
<td><strong>Biomarkers</strong></td>
<td></td>
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<tr>
<td>Urine Hex4, mmol/mol</td>
<td>4.6 ± -1.9 (0.3)</td>
<td>6.9 ± 1.2 (0.7)</td>
</tr>
<tr>
<td>Serum CK, U/L</td>
<td>447.0 ± -130.5 (25.1)</td>
<td>527.8 ± 60.2 (26.2)</td>
</tr>
</tbody>
</table>

Based on LOCF means

- Treatment group favored
- Nominal statistical significance ($p<0.05$)
Phase 3 PROPEL Study Results
Overall Population (n=122*)

Primary and First Key Secondary Endpoint Showed Greater Improvement with AT-GAA vs. alglucosidase alfa in the Overall Population of ERT-Naive and ERT-Experienced Patients

6MWD = 6-minute walk distance; FVC = forced vital capacity; SE = standard error. P values are nominal 2-sided; FVC data normally distributed and P value is from ANCOVA. 6MWD data not normally distributed and P value is for nonparametric ANCOVA.

*Results exclude one outlier subject
**Phase 3 PROPEL Topline Results:**
ERT Experienced Population (n=95)

ERT Experienced Patients Treated with AT-GAA Demonstrated Improvements over Time in 6MWD and Stabilization over Time in FVC Versus alglucosidase alfa

**6MWD (m): Change from baseline**
(n=65, n=30)

- **Mean(SE) Actual Change in 6MWD (m)**
  - Baseline
  - Week 12
  - Week 26
  - Week 38
  - Week 52 LOCF

**FVC (% predicted): Change from baseline**
(n=65, n=30)

- **Mean(SE) Actual Change in FVC (% predicted)**
  - Baseline
  - Week 12
  - Week 26
  - Week 38
  - Week 52 LOCF

**NOTE:** Baseline is Mean (STDEV); CFBL is Mean (SE); P-values are nominal 2-sided; FVC data normally distributed and p-values are from ANCOVA; 6MWD data not normally distributed and 6MWD p-value is for non-parametric ANCOVA; 6MWD parametric MMRM p-value was p=0.078
Phase 3 PROPEL Study Publication

Clinically Meaningful Outcomes from Phase 3 PROPEL Study Provide the Basis for Global Regulatory Submissions of AT-GAA

Peer-reviewed results from PROPEL suggest that treatment with AT-GAA provided clinically meaningful improvements over standard of care, including ERT-experienced patients with high unmet need.

The authors deemed AT-GAA to provide a differentiated mechanism of action and potential alternative treatment option for people living with late-onset Pompe Disease.
Long-Term Data from Phase 1/2 Clinical Study (ATB200-02)

Persistent and Durable Improvements in Motor and Respiratory Function and Reductions in Biomarkers of Muscle Damage and Disease Substrate Observed in Patients out to 36 Months

NOTE: * One patient in the ERT-naïve cohort experienced a large drop in % predicted FVC at month 21, which returned to previous levels at the following visit (month 24).
Global Pompe Disease Market Growth Continues to be Driven by the Diagnosis of New Patients - Only One Approved Therapy on the Market up until 2021

- Pompe Disease believed to be significantly underdiagnosed
  - Newborn screening studies suggest Pompe to be more prevalent than medical literature suggest (~1:10,000 to ~1:30,000)
  - Newborn screening already occurring in 27 U.S. states with 9 additional U.S. states pursuing NBS implementation for Pompe disease

Global Pompe Market to exceed $1.1B in 2021 and tracking toward $1.5B+ by 2025

1Global market measured by reported sales of approved therapies for Pompe disease – 2025 sales projected using 8% CAGR
AT-GAA: Key Takeaways

Focused on Advancing AT-GAA to as Many Patients as Possible through Global Regulatory Pathways and Expanded Access Mechanisms

- Regulatory status update:
  - U.S. PDUFA date 2H2022
  - CHMP opinion late 2022
  - Planning for additional regulatory submissions

- Multiple expanded access mechanisms in place, including in the U.K., Germany, France, Japan, and others

- 150+ people living with Pompe disease are on AT-GAA today across our clinical extension studies and expanded access programs

- Ongoing supportive studies:
  - Late-Onset Pompe Disease (LOPD) in children and adolescents aged 0 to <18
  - Infantile-Onset Pompe Disease (IOPD)

\(^1\)FDA PDUFA date of August 29, 2022 for miglustat NDA and October 29, 2022 for cipaglucosidase alfa BLA
Launch Preparations

Experienced and Passionate Rare Disease Medical and Commercial Organization Poised for Second Successful Launch

**Team**
- Highly leverageable team in place, few new hires needed
- Great experience and passion
- Eagerness to introduce a potential new therapy upon approvals

**Education**
- Published Phase 3 PROPEL data in The Lancet Neurology
- Active medical conference and publication schedule
- Continued education on biology of disease and diagnosis

**Access**
- Commitment to patient access
- Multiple Expanded Access Programs in place
- Demonstrating value to payors including parity pricing strategy

**Planning**
- Clear focus on launch
- Identification of key Pompe disease treatment centers
- Development of educational materials

**Key Strengths**
Financial & Operational Strategy
... maintaining a strong financial outlook
Revenue Performance

Q1 Revenue Growth of +18.5% to $79M resulting from Strong Operational Growth of +23.5% at CER Offset by Negative FX impact of -5.0%

- Year-over-Year Sales Growth
  - Significant currency exposure as 69% of Galafold revenue generated outside the U.S.
  - Applying average April 2022 exchange rates, the negative FX impact on full-year 2022 Galafold® reported sales would be approximately -6%
Financial Outlook and Path to Profitability

Clear Strategy to Build our Business, Advance our Portfolio, and Achieve Profitability

Sustain Galafold Revenue Growth

$79M 1Q2022 revenue, +24% YoY Operational Growth

2022 Galafold revenue guidance of $350M-$365M at CER, +15-20% YoY Growth

Secure Approvals of AT-GAA

Galafold and AT-GAA expected to drive strong double-digit growth long term

Deliver on Financial Goals

Focused on prudent expense management

2022 Non-GAAP operating expense guidance of $470M-$485M

Achieve profitability\(^1\) in 2023

\(^1\)Based on projections of Amicus non-GAAP Net Income under current operating plans, which includes successful AT-GAA regulatory approvals and continued Galafold growth. We define non-GAAP Net Income as GAAP Net Income excluding the impact of share-based compensation expense, changes in fair value of contingent consideration, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, loss on impairment of assets, restructuring charges and income taxes.
True Measure of Success: Impacting the Lives of Patients Living with Rare Diseases

YE17

>350 Patients*

YE21

>1,900+ Patients*

2023+

Thousands of Patients*

*Clinical & commercial, all figures approximate
Thank You
**Environmental, Social, & Governance (ESG) Snapshot**

### Who We Serve
Programs we invest in have 3 key characteristics:
- Address a rare genetic disease
- First-in-class or best-in-class
- Impart meaningful benefit for patients

**Pledge for a Cure**
Designate a portion of product revenue back into R&D for that specific disease until there is a cure.

**Pricing PROMISE**
Committed to never raising the annual price of our products more than consumer inflation.

**Charitable Giving**
Contributions allocated:
- **$1,677,000** US
- **$832,976** Intl.

**Expanded Access through 2021:**
- 52 patients / 18 countries

- Amicus supported community programs: 20+
- Volunteer hours (US): 770

**Global Employees**
- 496
- % female employees: 58%
- % Hiring Slate Diversity: 82%

### Diversity, Equity & Inclusion (DEI)
**2023 and Beyond:**
- Maintain strength in global gender diversity
- Increase US diversity through intentional and ongoing action
- Continuously evaluate compensation practices to ensure pay parity

### Environmental Management
Eco-friendly decision-making has unearthed economic efficiencies while continuing to bolster our standing as a good corporate citizen.

### Employee Recruitment, Engagement, and Retention
Leverage employee capabilities and expertise to provide a culture that drives performance and ultimately attracts, energizes, and retains critical talent.

Pulse surveys reveal employees feel **high personal satisfaction** in their job, are **proud of their work** and what they contribute to the community.

### Board of Directors
Committed to ongoing Board refreshment and diversity of background, gender, skills, and experience:
- **Director Diversity**
  - 3 Female
  - 2 Veteran Status
  - 1 African American
- **Board Independence**
  - 82%
- **Overall Board Diversity**
  - 55%

### Career Development
Reimagined performance management process to measure the what and the how, rewarding those who role-model our **Mission-Focused Behaviors**.
### Amicus Therapeutics, Inc.

#### Reconciliation of Non-GAAP Financial Measures

(in thousands)

<table>
<thead>
<tr>
<th></th>
<th>Three Months Ended</th>
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<tr>
<td></td>
<td>March 31,</td>
<td>2022</td>
<td>2021</td>
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<tr>
<td><strong>Total operating expenses - as reported GAAP</strong></td>
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<tr>
<td><strong>Research and development</strong></td>
<td></td>
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<tr>
<td>Share-based compensation</td>
<td>$ 146,472</td>
<td>$ 112,918</td>
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<tr>
<td><strong>Selling, general and administrative</strong></td>
<td></td>
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<tr>
<td>Share-based compensation</td>
<td>9,365</td>
<td>6,305</td>
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<tr>
<td>Loss on impairment of assets</td>
<td>21,286</td>
<td>14,049</td>
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<tr>
<td>Changes in fair value of contingent consideration payable</td>
<td>(1,188)</td>
<td>471</td>
<td></td>
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<tr>
<td><strong>Depreciation and amortization</strong></td>
<td>1,411</td>
<td>1,604</td>
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<tr>
<td><strong>Total operating expense adjustments to reported GAAP</strong></td>
<td></td>
<td>37,490</td>
<td>22,429</td>
</tr>
<tr>
<td><strong>Total operating expenses - as adjusted</strong></td>
<td></td>
<td>$ 108,982</td>
<td>$ 90,489</td>
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