BioMarin Pharmaceutical Inc.

Global Leader in Rare Disease Therapeutic Discovery, Development and Commercialization
Safe Harbor Statement

This non-confidential presentation contains ‘forward-looking statements’ about the business prospects of BioMarin Pharmaceutical Inc., including potential future products in different areas of therapeutic research and development. Results may differ materially depending on the progress of BioMarin’s product programs, actions of regulatory authorities, availability of capital, future actions in the pharmaceutical market and developments by competitors, and those factors detailed in BioMarin’s filings with the Securities and Exchange Commission such as 10-Q, 10-K and 8-K reports.
Our Mission:

We develop therapeutics that have a real impact for patients who live with **serious** and **life-threatening rare genetic diseases**

We remain committed to bringing new treatments to market that will provide a **meaningful benefit** to **small and medium-sized patient populations**
Our Challenge: We seek to **even the odds** for patients with rare genetic diseases

There are **over 7,000** identified rare diseases

~**30mm patients** diagnosed in the U.S., 50% are children

Only ~**7%** of rare diseases currently have treatments

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"I’m enjoying what life has to offer and proving big things do come in small packages. I want the world to know that "Differences Are Blessings". Thanks for helping me pursue my passion of singing and living life to the fullest, and congratulations on 30 years!"

**ALENA CARLY GALAN**

"Thank you for giving me a chance to run and play with George."

**BELLA BURTON**

"My experiences with my team at BioMarin are pretty worry free. Any issues I’ve ever had, they work with insurance and anyone else to get everything figured out. It’s amazing how all I have to do is send a text message, and so many people spring into action to make things happen. Thank you for all that you do!"

**BAILEY FLEMING**

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**MPS VI**

**Naglazyme**

(GALSULFASE)

**MPS IVA**

**Vimizim**

(elosulfase alfa)

**PKU**

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Unique Combination of Strong Commercial Base Business and Proven R&D Engine

**Established Base Business**

- Low-risk base business that is growing towards ~$2 billion revenue by 2020
  - Base business growing ~15% annually
  - Base business products are treatments for life
  - Complex Manufacturing = Low Generic Risk
- Diversified product, patient and geographic base
  - 7 products marketed in a 78 country commercial footprint. No one product > 1/3 revenue

**Productive R&D Engine**

- 7 products developed to approval; 2 approvals in the last 2 years
- 2 major blockbuster products on the horizon over next 18 months
- Research engine producing new product candidates annually
- Demonstrated results partnering with health authorities on global approvals
- Manufacturing resources capable of producing complex Biologics and Gene Therapy
Our History: Our track record of success spans over two decades

Twenty Years, Seven Treatments...

- 1997: BioMarin is Incorporated
- 1999: First clinical trial for Aldurazyme initiated
- 1999: Naglazyme approved by the FDA
- 1999: First clinical trial for Naglazyme initiated
- 2001: Naglazyme (GalNAc-SF) IND filed
- 2003: Aldurazyme approved by the FDA & EMA, becoming the first treatment for an MPS condition in the world
- 2003: Acquires Glyko Biomedical
- 2005: Kuvan approved by the FDA
- 2005: Palynziq Phase 1/2 initiated
- 2006: Firdapse launched in the EU
- 2007: Vimizim approved by the FDA
- 2007: Brineura clinical program announced
- 2009: Vosoritide first clinical trial initiated
- 2011: First patient enrolled in Valrox Phase 1/2
- 2013: First patient enrolled in Phase 1/2 clinical trial for BMN250 in MPS IIIB
- 2015: Vosoritide Brineura Phase 1/2 clinical trial initiated
- 2015: Vimizim approved by the FDA
- 2017: Brineura approved
- 2019: Palynziq approved

First patient enrolled in Phase 1/2 clinical trial for BMN250 in MPS IIIB
First patient enrolled in Valrox Phase 1/2
Vosoritide Brineura Phase 1/2 clinical trial initiated
Vimizim approved by the FDA
Brineura approved

...Thousands of Lives Improved
Our Approach: We attack rare disease where it begins – at the genetic level

Enzyme Replacement Therapy

Gene Therapy

7 Approved Products
- Palynziq (PKU)
- Vimizim (MPS IVA)
- Kuvan (PKU)
- Naglazyme (MPS VI)
- Aldurazyme (MPS I)
- Brineura (Batten Disease)
- Firazyr (LEMS)

Wholly-Owned Clinical Pipeline
- Valrox (Phase 3) - Hemophilia A
- Vosoritide (Phase 3) - Achondroplasia
- BMN 250 (Phase 2) - MPS IIIB

Drug Discovery Engine
- PKU Gene Therapy - Preclinical
- Gene Therapy & Next-Gen ERT
Organization Overview
We are a Global, Fully Integrated Rare Disease Leader

High-Science, Innovative Approach to Drug Discovery
- 5 of 7 current marketed products were developed in-house
- 4 of 4 pipeline products were discovered in-house
- 500+ of 2,500 have PhD or equivalent degree

Highly Efficient and Effective R&D Machine with Strong Regulatory Capabilities
- Average 5 years from IND filings to approval
- Global leader in bringing products to market for orphan diseases
- >70% success rate in pivotal studies

Best-in-Class Commercial Manufacturing Capabilities
- 2 wholly-owned cGMP biologics facilities which currently manufacture 5 BioMarin products
- First, and largest, GT manufacturing facility
- Accelerates clinical development and supports regulatory activities to meet expected commercial demand

Commercial infra-structure supporting seven approved therapies targeting a range of orphan diseases, including:
- PKU
- Batten’s Disease
- Morquio A
- MPS VI
- MPS I
- LEMS
Efficient R&D Engine has Produced a Broad Marketed Product Portfolio

5 years on average from IND to approval for all marketed products

- First Ever treatment for MPS Disease
- First Sulfatase Treatment For MPS Disease
- First Chaperone Treatment for PKU
- First Treatment for MPS IVA Disease
- First Enzyme Chronically Delivered by ICV Route
- First Bacterial Enzyme Chronically Delivered by SQ Route

Innovation Creativity Across Technology

Source: Company presentations and filings
Wholly Owned Manufacturing Supports Rapid Drug Development and Commercialization

Biologics Facilities

**Novato, California**
- Opened: 1999
- Footprint: 80,000ft²
- Manufactures: Aldurazyme, Brineura, Naglazyme, Palynziq, Vimizim, Vosoritide

**Shanbally, Ireland**
- Opened: 2017
- Footprint: 200,000ft²
- Manufactures: Brineura, Vimizim, BMN 250

Gene Therapy Facility

**Novato, California**
- Commissioned: 2017
- Footprint: 50,000ft²
- Manufactures: Valrox, BMN 307 PKU GT
- Utilizes Multiple 2000L Bioreactors, Capable of Supporting 4,000-5000 Patients / Year

Substantial track record of inspections and cGMP compliance

Source: Company presentations and filings
Our Global Scope Allows Us to Expand Access for Patients and Maximize the Commercial Potential of Our Products

Note: Figures represent 2018A Net Sales
Commercial Organization  
Base Business Continues to Thrive
Consistent Commercial Execution

Sales Have Beat Consensus Expectations in 78% of Quarters Since 2017
7 Approved Products Targeted to Deliver $2B in Revenue in 2020

Commercialized Products

- Palynziq
- Brineura
- Vimizim
- Naglazyme
- Kuvan
- Aldurazyme + Other

(Revenue in millions)

2019 FY Guidance
$1,680 - $1,750

Commercial base business expected to drive 15%+ top-line growth Y/Y in 2019
Our Most Recently Approved Product

- U.S. launch drove 551 commercial patients on Palynziq at end of 2Q19
- An additional 158 are enrolled and awaiting their first treatment
- 2019 FY revenues expected to be between $70M-$100M
Palynziq Approved in the U.S. May 2018; Strong launch Underway

Commercial Progress:

- FY19 Palynziq revenue guidance $70-$100 million
- Average annual price of $192,000 per patient, reached after ~6 months stepwise titration dosing
- >700 Palynziq patients enrolled, as of the end of 2Q19, representing a run-rate from U.S. patients of ~$130 million annual revenue

PKU Patients ≥18 years in U.S.

<table>
<thead>
<tr>
<th>Category</th>
<th>Number</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total:</td>
<td>11,700</td>
</tr>
<tr>
<td>On Kuvan</td>
<td>~7,500</td>
</tr>
<tr>
<td>On Palynziq</td>
<td>~1,400</td>
</tr>
<tr>
<td>In Clinic</td>
<td>~600</td>
</tr>
<tr>
<td>Out of Clinic</td>
<td>~2,200</td>
</tr>
</tbody>
</table>

PKU patients defined as patients diagnosed through newborn screening; Out-of-clinic patients are those who have been diagnosed, but have not returned to clinic in at least 2 years.
PKU represents BioMarin’s largest U.S. patient population opportunity
>11,000 adult patients with PKU, 3,900 actively managed in-clinic

Breadth and depth of adoption across key clinics, both clinical trial sites and naïve clinics

- Sites with ≥1 complete enrollment: 89
- Patients on reimbursed Palynziq: 551
  - Clinical Study Patients: 141
  - Formerly Naïve Patients: 410
- Patients enrolled but not yet reimbursed/on therapy: 158

Positive payer coverage at launch leading to strong uptake of reimbursed patients

Leading indicators point toward continued uptake acceleration in 2019
Palynziq Approved in the EU May 2019; Launch Underway 2H19

Market Attributes:

- Large initial commercial market of 5,800 in-clinic PKU patients aged 16 and older
- 3 years of direct experience working with PKU community to prepare for launch
- Anticipate meaningful revenue in EU starting 2020 following usual pricing and reimbursement process by country

PKU Patients ≥16 years in EUMEA

- Total: 18,000
- ~5,800 in-clinic
- ~11,700 out of clinic
- ~500 on Kuvan

PKU patients defined as patients diagnosed through newborn screening; EUMEA includes Europe, Turkey, Russia and Middle East.
Our Next Phase 3 Readout for Potential Blockbuster

Vosoritide
(achondroplasia)

- Global Phase 3 enrolled; data expected YE 2019
- 0-5 year-old study underway:
  - Cohort 1 (24-60 month olds) enrollment complete
  - Cohort 2 (6-24 month olds) enrollment expected to complete by year-end
  - Cohort 3 (newborn-6 month olds) enrollment to begin by year-end
What is Vosoritide?

- Vosoritide binds to a specific receptor, which initiates intracellular signals that inhibit overactive FGFR3. FGFR3 inhibition encourages cartilage and bone growth for achondroplasia, the most common form of dwarfism.

Why is Vosoritide Important?

- No approved treatments except limb-lengthening surgery
- Serious disease complications include:
  - Foramen magnum compression
  - Sleep apnea
  - Bowed legs
  - Permanent sway of the lower back
  - Spinal stenosis
  - Obesity

Vosoritide – Potential First-to-Market Treatment for Achondroplasia

✓ Sustained increase in growth velocity for up to 42 months
✓ Well tolerated safety profile
✓ Global Phase 3 enrolled, data expected YE 2019

Achondroplasia Overview

- Achondroplasia is caused by a mutation in the fibroblast growth receptor 3 gene (FGFR3), a negative regulator of bone growth.
- The condition occurs in 1 in 15,000-40,000 newborns worldwide
- ~20,000 children with achondroplasia in BioMarin’s global territories
Growth Characteristics in Achondroplasia

Children with Achondroplasia Grow an Average of 4 cm/year vs. 6 cm/year for Average Height Children
Durable Growth Sustained through 42-months with Vosoritide 15µg/kg Dose

42 Month Additional Height Gained is 5.7cm with Vosoritide

Sustained elevation of AGV shown in sequential 6-month time periods in ongoing Phase 2 study
Executing Strategy to Demonstrate Improved Clinical Outcomes with Vosoritide

Comprehensive global program designed to include children from birth to final adult height

**Phase 3**
- 2 year, placebo-controlled, 121 subjects, 52 weeks of treatment after 52 weeks of baseline observation
- 90% powered to detect statistically significant differences between treatment arm and placebo arm
- Data end 2019/start 2020

**Phase 2 (5 to 14 years)**
- Demonstrated additional height gain of 5.7cm over 42 months
- Generally well-tolerated with no hypotension signal. Over 30,000 injections administered and only one event of symptomatic hypotension
- 54 month data before year-end 2019

**Phase 2 (0 to <5 years)**
- Cohort 1 (24-60 months) enrolled
- Cohort 2 (6-24 months) enrolling
- Cohort 3 (newborn-6 months) to begin by year-end
- Safety data potentially available at time of regulatory submissions

**Natural History Data**
- Multi-center, retrospective, prospective and cross-sectional study, N=1,377
- Comparisons between on treatment final adult height and Natural History data to support assessment of efficacy over 1 year

Next Steps:
- Phase 3 data read-out by year-end
- Phase 2 54-month update before year-end 2019
Valoctocogene Roxaparvovec (severe Hemophilia A) Latest guidance: BioMarin plans to submit marketing applications to both the FDA and the EMA in 4Q 2019
## Valrox for Hemophilia A: Phase 1/2 and Phase 3 Study Designs

<table>
<thead>
<tr>
<th>Phase 1/2 201 Study</th>
<th>Phase 3 GENER8-1 Study</th>
<th>Full Study</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>N</strong></td>
<td>7</td>
<td>20</td>
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<tr>
<td><strong>Study Duration</strong></td>
<td>3-years' data May 2019</td>
<td>26 weeks</td>
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<td>(5-year study)</td>
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<tr>
<td><strong>Endpoints</strong></td>
<td>Safety; ABR; annualized FVIII replacement therapy infusion rate; FVIII activity levels</td>
<td>Safety; FVIII activity levels</td>
</tr>
<tr>
<td><strong>Steroid Protocol</strong></td>
<td>Prophylactic at 3 weeks</td>
<td>On-demand at 10 weeks</td>
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<tr>
<td><strong>Results as of Last Update</strong></td>
<td>Bleed rate controlled for 3 years (median ABR=0, mean ABR=0.7); 96% reduction in both mean ABR &amp; mean annualized FVIII usage</td>
<td>40% achieved FVIII activity levels of 40 IU/dL or better; median/mean ABR was 0/1.5 (85% reduction from baseline levels where all patients were on SOC); 94% reduction in mean annualized FVIII usage in weeks 5-26</td>
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<tr>
<td><strong>Next Steps</strong></td>
<td>4-year data analysis</td>
<td>Submit marketing applications to FDA and EMA in 4Q19 with potential approval(s) in 2020</td>
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</table>
Phase 2 (3 year) and Phase 3 (.5 years) Results at Last Update

Consistent efficacy observed across Phase 2 and Phase 3 interim analysis cohorts

LOESS (LOcally Estimated Scatterplot Smoother) Curves of FVIII Activity Level Over Time
Later Initiation of Steroids in P3 Associated with Decreased FVIII Activity

Comparison of Phase 2 and Phase 3 steroid use

<table>
<thead>
<tr>
<th>Study Metrics</th>
<th>Phase 2: 201 Study</th>
<th>Phase 3: GENEr8-1 IA mITT</th>
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<tbody>
<tr>
<td>Mean/median time to steroid initiation (weeks)</td>
<td>3.0/3.1</td>
<td>10.9/10.0</td>
</tr>
<tr>
<td>ALT elevation* within first 26 weeks</td>
<td>5/7 (71%)</td>
<td>12/16 (75%)</td>
</tr>
<tr>
<td>ALT elevation* within first 26 weeks associated with decreased FVIII activity</td>
<td>1/5 (20%)</td>
<td>7/12 (58%)</td>
</tr>
</tbody>
</table>

Plan to continue Phase 3 study as designed, optimizing “on-demand” steroid use

- Increasing confidence in demonstrating improvement in ABR at week 52

MAA (EU) and BLA (US) on-track for submissions in 4Q19 with potential approval(s) in 2020

*Protocol defined as: Alt > 1.5 ULN; or ALT>2x baseline and ALT>ULN
Recent Survey: Speedy Uptake >40% Market Share in Severe Population in 5 Years

Hematologist Anticipated Valrox Usage

Overview – Survey Key Takeaways

- Hematologists anticipate robust uptake; among eligible severe hemophilia patients ~25% immediately after approval and ~45% after 5 years

Source: MEDACorp Survey; "Hemophilia", February 2019
Multiple Lifecycle Management Opportunities Exist to Expand Hemophilia A Market Opportunity

Goal to expand into additional Hemophilia A patient subpopulations over time

~30,000 patients
- AAV5 Ab free, > 18 y/o, severe

~50,000 patients
- AAV5 Ab+
- All prophy subjects
- Adolescents
  ✓ AAV5 Ab free, > 18 y/o, severe

> 70,000 patients
- All levels of severity
- All Ab-status
- All inhibitor status
  ✓ AAV5 Ab+
  ✓ All prophy subjects
  ✓ Adolescents
  ✓ AAV5 Ab free, > 18 y/o, severe

Valoctocogene Roxaparvovec
Initial label

Valoctocogene Roxaparvovec
Future label

~30,000 patients

~50,000 patients

> 70,000 patients
Global Hemophilia A Market in 2018 was $8.7B\textsuperscript{1}

Estimated WAC pricing for Hemlibra in non-inhibitor adult patients is between $600K-$800K per year\textsuperscript{2}

Fully-compliant, WAC pricing for FVIII replacement in adults is $403K-$674K per year\textsuperscript{3}

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<thead>
<tr>
<th>Region</th>
<th>Estimated Patients</th>
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<tr>
<td>NORAM</td>
<td>~20,500</td>
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<tr>
<td>LATAM</td>
<td>~20,400</td>
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<tr>
<td>EUMEA</td>
<td>~66,300</td>
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<tr>
<td>APAC</td>
<td>~11,500</td>
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An Estimated ~119K Hemophilia A Patients in our Territories

\textsuperscript{1} Evaluate Pharma 2019; \textsuperscript{2} PriceRx IHA Global insight Oct. 2015-Oct. 2016 (WAC price reflects cost of Factor VIII replacement for an adult on prophylaxis)
\textsuperscript{2} Based on WAC price of $99.20 per mg $482K/year for a 58 kg subject; Centers for Disease Control and Prevention (CDC) estimates the average adult American male weighs >90 kg
\textsuperscript{3} EPI Data from 2016 WFH Annual Survey; NHF website: http://www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeding-Disorders/Hemophilia-A/
Guidance and Summary
Financial Performance and Outlook

**Actual and Estimated Revenue 2005-2019**

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<tbody>
<tr>
<td>Palynziq</td>
<td>$26</td>
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<tr>
<td>Brineura</td>
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<td>$84</td>
<td>$122</td>
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<td>Vimizim</td>
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<td>Naglazyme</td>
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<td>Kuvan</td>
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<tr>
<td>Aldurazyme + Other</td>
<td></td>
<td>$297</td>
<td>$325</td>
<td>$376</td>
<td>$441</td>
<td>$501</td>
<td>$549</td>
<td>$751</td>
<td>$890</td>
<td>$1,117</td>
<td>$1,313</td>
<td>$1,491</td>
<td>$1,680</td>
<td>$1,750</td>
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**Full Year 2019 Revenue Guidance**

($ in millions)

<table>
<thead>
<tr>
<th>Item</th>
<th>Provided 2/21/19</th>
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</thead>
<tbody>
<tr>
<td>Total BioMarin Revenues</td>
<td>$1,680 to $1,750</td>
</tr>
<tr>
<td>Vimizim</td>
<td>$530 to $570</td>
</tr>
<tr>
<td>Kuvan</td>
<td>$420 to $460</td>
</tr>
<tr>
<td>Naglazyme</td>
<td>$350 to $380</td>
</tr>
<tr>
<td>Palynziq</td>
<td>$70 to $100</td>
</tr>
</tbody>
</table>

**Selected Income Statement Guidance**

($ in millions, except percentages)

<table>
<thead>
<tr>
<th>Item</th>
<th>Provided 2/21/19</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost of Sales(% of Total Rev.)</td>
<td>20% to 21%</td>
</tr>
<tr>
<td>R&amp;D Expense</td>
<td>$740 to $780</td>
</tr>
<tr>
<td>SG&amp;A Expense</td>
<td>$650 to $690</td>
</tr>
<tr>
<td>Non-GAAP Net Income</td>
<td>$130 to $170</td>
</tr>
<tr>
<td>GAAP Net Loss</td>
<td>$(45) to $(85)</td>
</tr>
</tbody>
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**Forward Guidance**

Revenue: Grow 15%/year to $2B in 2020, accelerated growth after that w/ Valrox & Vosoritide

Bottom Line: Non-GAAP + starting 2017, grow annually, accelerate GAAP profitability

No equity financings: Except for M&A or convert refinancing’s
Four Pillars of Growth Driving Value

Strong and Growing Base Business:
FY19 Revenues Expected to Grow ~15% Year over Year*/$2B in 2020

New Product Launches:
2Q19 Palynziq enrollments represent ~$130M run-rate in U.S. alone

Next Potential Products Represent Significant Addressable Markets
Vosoritide (~24K) and Valrox (~30K)

Manufacturing Capabilities to Support Commercial and R&D Growth
Expertise in small molecule, biologics & gene therapy production

* At the mid-point of current FY 2019 guidance
THANK YOU

BiOMARIN