May 28, 2019

Valoctocogene Roxaparvovec Phase 2 and Phase 3 Update

JJ Bienaime, Chairman and Chief Executive Officer
Hank Fuchs, President, Worldwide R&D
Safe Harbor Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including without limitation, statements about the development of BioMarin's valoctocogene roxaparvovec program generally, the possibility of an accelerated and/or conditional filing and approval by the FDA or EMA, respectively; the impact of valoctocogene roxaparvovec gene therapy for treating patients with severe hemophilia A, the potential for valoctocogene roxaparvovec to reduce or eliminate bleeds, reduce the number of Factor VIII infusions, improve the quality of life and the ongoing clinical programs generally. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: results and timing of current and planned preclinical studies and clinical trials of valoctocogene roxaparvovec, including final analysis of the above interim data and additional data from the continuation of these trials; any potential adverse events observed in the continuing monitoring of the patients in the clinical trials; the content and timing of decisions by the FDA, the EMA and other regulatory authorities; the content and timing of decisions by local and central ethics committees regarding the clinical trials; our ability to successfully manufacture the product candidate for the preclinical and clinical trials; and those other risks detailed from time to time under the caption "Risk Factors" and elsewhere in BioMarin's Securities and Exchange Commission (SEC) filings, including BioMarin's Annual and quarterly Reports on Forms 10-K and 10-Q, and future filings and reports by BioMarin. BioMarin undertakes no duty or obligation to update any forward-looking statements contained in this press release as a result of new information, future events or changes in its expectations.

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Key Highlights of Today’s Valrox News

Phase 3 interim analysis cohort efficacy data supports plans to submit marketing authorizations in both the US and EU

- Potential for YE 2020 US launch
- FVIII response level in subjects met pre-specified criteria established following discussion with FDA
- Dramatic reduction in mean ABR from 10 (on Standard of Care) to less than 1.5

Phase 2 Three-year ABR Update Results:
- Clinical benefit maintained with an ABR rate of <1 for 3 consecutive years in the 6e13 vg/kg dose and <1.5 for 2 consecutive years with the 4e13 vg/kg dose
- 96% reduction in mean ABR with the 6e13 vg/kg dose over 3 years in pre-treated subjects

Phase 2 Three-year Factor VIII Durability Update Results:
- Rate of FVIII decline continued to be expression level dependent, slowed in year 3, and appears to be approaching plateau
Rapid Development has Advanced Valrox from FPI to Potential Approval in <5 Years

2013
FVIII collaboration with UCL/St. Jude Children’s Research Hospital

2014
Selection of “valrox” for development

2015
CTA filed in EMA/September FPI in Phase 1/2

2016
Phase 1/2 continues; gene therapy facility built/producing commercial material

2017

2018
Phase 3 GENEr8-1 study enrollment begins; FDA Accelerated Approval guidelines published

2019
Phase 3 interim cohort results meets submission criteria; decision 3Q19; Potential approval 3Q20

2020
May 28, 2019

Valoctocogene Roxaparvovec “valrox” Phase 2 Update

Hank Fuchs, President, Worldwide R&D
Substantial Reduction in Treated Annualized Bleed Rate (ABR) with Both Doses

6e13 vg/kg dose cohort at Years 1, 2 & 3; N=6*

<table>
<thead>
<tr>
<th>ABR (episodes/year)</th>
<th>Pre-infusion</th>
<th>Y1 post</th>
<th>Y2 post</th>
<th>Y3 post</th>
</tr>
</thead>
<tbody>
<tr>
<td>median</td>
<td>16.5</td>
<td>0.9</td>
<td>0.2</td>
<td>0.7</td>
</tr>
<tr>
<td>mean</td>
<td>16.3</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

96% reduction in mean ABR

4e13 dose cohort at Years 1 & 2; N=6

<table>
<thead>
<tr>
<th>ABR (episodes/year)</th>
<th>Pre-infusion</th>
<th>Y1 post</th>
<th>Y2 post</th>
</tr>
</thead>
<tbody>
<tr>
<td>median</td>
<td></td>
<td>0.9</td>
<td></td>
</tr>
<tr>
<td>mean</td>
<td></td>
<td>1.2</td>
<td></td>
</tr>
</tbody>
</table>

92% reduction in mean ABR

* The one subject treated with on demand rFVIII pre-infusion was excluded

% Patients Bleed Free**

<table>
<thead>
<tr>
<th></th>
<th>Baseline</th>
<th>Year 1</th>
<th>Year 2</th>
<th>Year 3</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>14%</td>
<td>71%</td>
<td>86%</td>
<td>86%</td>
</tr>
</tbody>
</table>

All patients off prophylaxis; 100% resolution in target joints

% Patients Bleed Free

<table>
<thead>
<tr>
<th></th>
<th>Baseline</th>
<th>Year 1</th>
<th>Year 2</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>17%</td>
<td>83%</td>
<td>67%</td>
</tr>
</tbody>
</table>

All patients off prophylaxis

** N=7 and includes the one subject treated with on demand rFVIII pre-infusion
Valrox Phase 2 Subjects Demonstrate Continued Reduction in FVIII Usage

**6e13 vg/kg dose cohort FVIII usage in years 1, 2 & 3; N=6**

<table>
<thead>
<tr>
<th>Annualized FVIII Usage (infusions/year)</th>
<th>Pre-infusion</th>
<th>Y1 post</th>
<th>Y2 post</th>
<th>Y3 post</th>
</tr>
</thead>
<tbody>
<tr>
<td>Median</td>
<td>138.5</td>
<td>0</td>
<td>8.8</td>
<td>5.5</td>
</tr>
<tr>
<td>Mean</td>
<td>136.7</td>
<td>2.1</td>
<td>0</td>
<td>0</td>
</tr>
</tbody>
</table>

96% reduction in mean FVIII usage

**4e13 vg/kg dose cohort FVIII usage in years 1 & 2; N=6**

<table>
<thead>
<tr>
<th>Annualized FVIII Usage (infusions/year)</th>
<th>Pre-infusion</th>
<th>Y1 post</th>
<th>Y2 post</th>
</tr>
</thead>
<tbody>
<tr>
<td>Median</td>
<td>155.5</td>
<td>2</td>
<td>0.5</td>
</tr>
<tr>
<td>Mean</td>
<td>146.5</td>
<td>6.8</td>
<td>6.8</td>
</tr>
</tbody>
</table>

97% reduction in mean FVIII usage

<table>
<thead>
<tr>
<th>Reduction in FVIII usage post valrox 6e13 dose</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year 1</td>
</tr>
<tr>
<td>Mean</td>
</tr>
<tr>
<td>Median</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Reduction in FVIII usage post valrox 4e13 dose</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year 1</td>
</tr>
<tr>
<td>Mean</td>
</tr>
<tr>
<td>Median</td>
</tr>
</tbody>
</table>

* The one subject treated with on demand rFVIII at baseline was excluded
3 Year Phase 2 6e13 vg/kg Data Demonstrates Durable Factor VIII Expression

Rate of FVIII decline continued to be expression level dependent, slowed in year 3, and appears to be approaching plateau

<table>
<thead>
<tr>
<th>FVIII activity level (IU/dL) time point</th>
<th>Mean (Chromogenic)</th>
<th>Median (Chromogenic)</th>
<th>Mean (One Stage)</th>
<th>Median (One Stage)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Weeks 23-26</td>
<td>68</td>
<td>57</td>
<td>127</td>
<td>100</td>
</tr>
<tr>
<td>Week 52</td>
<td>64</td>
<td>60</td>
<td>104</td>
<td>89</td>
</tr>
<tr>
<td>Week 104</td>
<td>36</td>
<td>26</td>
<td>59</td>
<td>46</td>
</tr>
<tr>
<td>Week 156</td>
<td>33</td>
<td>20</td>
<td>52</td>
<td>30</td>
</tr>
</tbody>
</table>

Three year results with Chromogenic Assay
2 Year 4e13 vg/kg Dose Also Shows Durable Expression

<table>
<thead>
<tr>
<th>FVIII activity level (IU/dL)</th>
<th>Mean (Chromogenic)</th>
<th>Median (Chromogenic)</th>
<th>Mean (One Stage)</th>
<th>Median (One Stage)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Week 52</td>
<td>21</td>
<td>23</td>
<td>31</td>
<td>32</td>
</tr>
<tr>
<td>Week 104</td>
<td>15</td>
<td>13</td>
<td>23</td>
<td>24</td>
</tr>
</tbody>
</table>
FVIII Expression Reasonably Likely to Predict Hemostatic Efficacy

Advent of bleeding events by FVIII activity levels during discreet 4 week intervals in all subjects
Rate of Factor VIII Change in Phase 2 is Level Dependent
The Lower the Factor VIII Level, the Lower the Rate of Change

Factor VIII Year over Year Changes:

<table>
<thead>
<tr>
<th>Year</th>
<th>Mean (IU/dL)</th>
<th>Change (IU/dL)</th>
<th>% Change</th>
<th>Median (IU/dL)</th>
<th>Change (IU/dL)</th>
<th>% Change</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year 1</td>
<td>64.3</td>
<td>60.3</td>
<td>60.3</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Year 2</td>
<td>36.4</td>
<td>-27.9</td>
<td>-43%</td>
<td>26.2</td>
<td>-34.1</td>
<td>-57%</td>
</tr>
<tr>
<td>Year 3</td>
<td>32.7</td>
<td>-3.7</td>
<td>-10%</td>
<td>19.9</td>
<td>-6.3</td>
<td>-24%</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Year</th>
<th>Mean (IU/dL)</th>
<th>Change (IU/dL)</th>
<th>% Change</th>
<th>Median (IU/dL)</th>
<th>Change (IU/dL)</th>
<th>% Change</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year 1</td>
<td>21.0</td>
<td>22.9</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Year 2</td>
<td>14.7</td>
<td>-6.3</td>
<td>-30%</td>
<td>13.1</td>
<td>-9.8</td>
<td>-43%</td>
</tr>
</tbody>
</table>

Advent of bleeding events by FVIII activity levels during discreet 4 week intervals in all subjects
Durability Modeled Based on Observed Expression Level Dependence

Solid lines represent a cubic spline fit through the available FVIII Activity data through 156 weeks (Study 201; 6E13 vg/kg) and 104 weeks (Study 201; 4E13 vg/kg).

Dashed lines represent estimated slopes from linear regressions of the observed FVIII activity data from > 52 weeks to ≤ 156 weeks (Study 201; 6E13 vg/kg) and from > 52 weeks to ≤ 104 weeks (Study 201; 4E13 vg/kg) with 95% confidence intervals.

Extrapolation beyond week 156 (Study 201; 6E13 vg/kg) assumes slope is equal to slope estimated for the 6E13 vg/kg dose before FVIII activity reaches 4e13 vg/kg levels at Year 2, and equal to slope estimated for 4E13 vg/kg thereafter.

Based on our interpretation of the 3 year data, we believe valrox is approaching the expected plateau seen with other AAV gene therapies.
Valrox Safety Profile Remains Very Favorable

• Valrox was well-tolerated with favorable safety profile; currently:
  • Transient, asymptomatic ALT elevation was observed in 6/7 (85.7%) subjects; all were grade 1, except for one that was grade 2
  • ALT levels within normal limits in all subjects
  • All subjects remain off corticosteroids
  • No inhibitors to FVIII
Phase 2 Conclusions with 6e13 vg/kg and 4e13 vg/kg Doses

Phase 2 Three-year ABR Update Results:
- Clinical benefit maintained with an ABR rate of <1 for 3 consecutive years in the 6e13 vg/kg dose and <1.5 for 2 consecutive years with the 4e13 vg/kg dose
- 96% reduction in mean ABR over 3 years in pre-treated subjects

Phase 2 Three-year Factor VIII Durability Update Results:
- Rate of FVIII decline continued to be expression level dependent, slowed in year 3, and appears to be approaching plateau
MAY 28, 2019

VALOCTOCOGENE ROXAPARVOLVEC “VALROX” PROGRAM UPDATE

PHASE 3 INTERIM ANALYSIS COHORT RESULTS WITH 6E13 VG/KG DOSE

HANK FUCHS, PRESIDENT, WORLDWIDE R&D
Phase 3 Interim Analysis Cohort met Criteria based on FVIII Activity Levels

• Pre-specified primary endpoint for registration was the proportion of patients whose FVIII levels were ≥ 40 IU/dL after discussion with FDA
  • At the April 30, 2019 data-cut, of the 16 subjects who had reached 26 weeks, 7 had median factor VIII levels ≥ 40 IU/dL during weeks 23-26.
  • An 8th subject met the pre-specified criteria after the April 30, 2019 data-cut
  • 3 more subjects still to be evaluated

• Observed FVIII expression levels and associated breakthrough bleeding indicate FVIII expression reasonably likely to predict hemostatic efficacy

• Based on discussions with EU regulatory authorities under the Priority Medicines Prime Regulatory Initiative, the interim analysis cohort data can also be used to support an EU filing application

• The Company intends to file BLA/Marketing Authorization applications in both the US and EU
  • Next steps include pre-submission meetings with health authorities in the US and EU so BMRN can determine timing of filings
Substantial Reduction in ABRs and FVIII Usage in Phase 3 Interim Analysis

85% reduction in mean ABR from baseline levels where all patients were on standard of care prophylaxis

95% reduction in mean FVIII usage annualized after week 5

**6e13 vg/kg dose Interim Analysis cohort**

- **ABR (episodes/year)**
  - Pre-infusion: 0.9, 2, 4, 6, 8, 10, 12
  - Post-infusion: 0, 1.5, 2, 4, 6, 8, 10
  - Median: 0.9, 0
  - Mean: 9.9, 1.5

- **Annualized FVIII Usage (infusions/year)**
  - Pre-infusion: 132.7, 146.1
  - Post-infusion: 1.2, 6.8
  - Median: 132.7, 1.2
  - Mean: 146.1, 6.8
Phase 3 Interim Analysis FVIII Activity Levels

mITT (N=16)

Results through 26 week results with Chromogenic Assay

Factor VIII Activity (IU/dL)

Study Weeks

1-4 5-8 9-12 13-16 17-20 21-24 23-26

N: 13 15 16 16 16 16 16

Mean Median
Hemostatic Efficacy Expected to Exceed 8 Years Based on Phase 2 Factor VIII Expression Model
Summary of Today’s News

• Phase 3 initial cohort data supports marketing authorization applications in both the US and EU

• Consistent with Phase 2 results, Phase 3 initial results demonstrated dramatic reduction in bleeding rate from mean ABR of 10 (on prophylaxis standard of care) to less than 1.5 ABR after 5 weeks from valrox infusion.

• Phase 2 3 year results demonstrated a maintained clinical benefit with an ABR rate of <1 for 3 consecutive years in the 6e13 vg/kg dose and <1.5 for 2 consecutive years with the 4e13 vg/kg dose; 96% reduction in mean ABR with the 6e13 vg/kg dose over 3 years in pre-treated subjects

• Rate of FVIII decline with valrox continued to be expression level dependent and slowed in year 3 of the 6e13 vg/kg and was even lower in year 2 of the 4e13 vg/kg

• Hemostatic Efficacy Expected to Exceed 8 Years Based on Phase 2 Factor VIII Expression Model
MAY 28, 2019

VALOCTOCOGENE ROXAPARVOVEC “VALROX” PROGRAM UPDATE

THANK YOU

Q&A