May 22, 2018 IR Call

World Federation of Hemophilia Congress
Valoctocogene Roxaparvovec program update

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 impact of valoctocogene roxaparvovec gene therapy for treating patients with severe hemophilia A, the potential for valoctocogene roxaparvovec to bring Factor VIII levels to normal, near normal or mild, and to reduce or eliminate bleeds, reduce the number of Factor VIII infusions, improve the quality of life, the planned Phase 3 clinical program, the ongoing Phase 1/2 study, Phase 1/2 study in people with AAV5+, or other possible future clinical studies of valoctocogene roxaparvovec. 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; any potential adverse events observed in the continuing monitoring of the patients in the Phase 1/2 trial; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, 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.

Welcome and Introduction

Geoff Nichol, Senior Vice President, Chief Medical Officer and Head of Global Clinical Development
Stuart bunting, Fellow, Translational Biology
Barrie Carter, Vice President, Vector biology
hank fuchs, President, Worldwide R&D

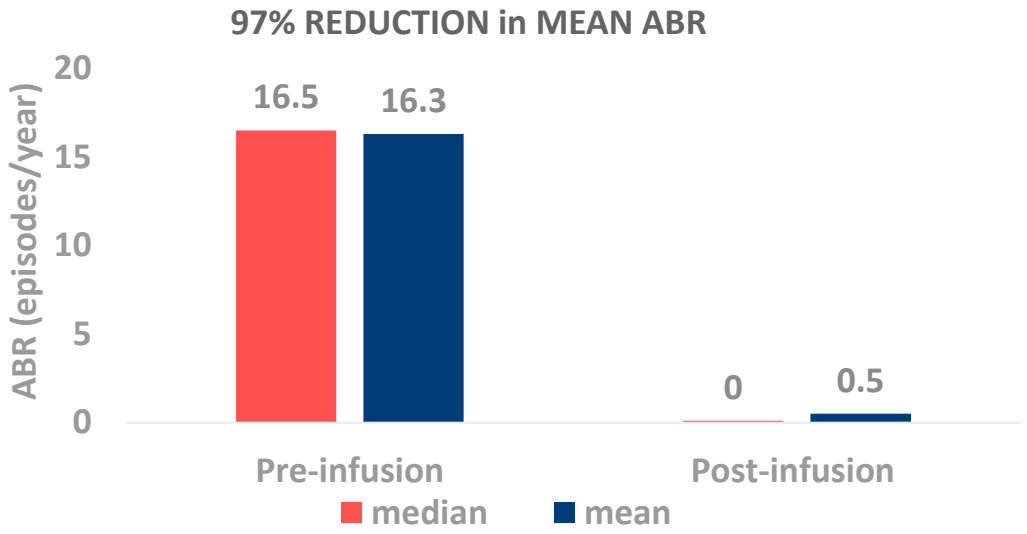
BMN 270 FAVOURABLE SAFETY PROFILE AS OF 16 APRIL 2018

- Transient transaminitis
  - 73% of subjects; now all resolved
    - 93% Grade 1, 7 % Grade 2 ALT elevation
  - Median onset at 7.6 weeks; duration $\leq$25.3 weeks post onset
- All subjects are off corticosteroids
- No subject developed inhibitors to FVIII; no subject withdrew
- 2 SAEs, both self-limited
  - Pyrexia, resolved overnight
  - Total knee replacement for pre-existing arthropathy
- Most common other AEs across all dose cohorts:
  - Arthralgia (60%); headache (47%), back pain (40%)
  - Viral upper respiratory tract infection (40%), fatigue, insomnia, pain in extremity (33%)

SUBSTANTIAL REDUCTION IN TREATED ANNUALIZED BLEED RATE (ABR)

STARTING FROM 4 WEEKS POST-INFUSION

6e13 dose through week 104



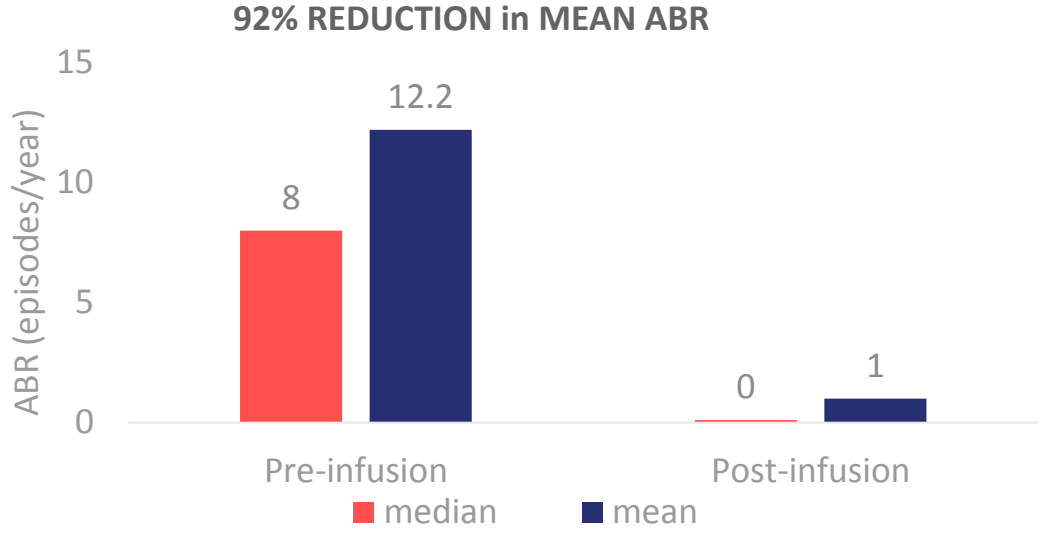
% Patients Bleed Free

Baseline	Year 1	Year 2
14%	71%	86%

All patients off prophylaxis

100% resolution in target joints

4e13 dose through week 52



% Patients Bleed Free

Baseline	Year 1
17%	83%

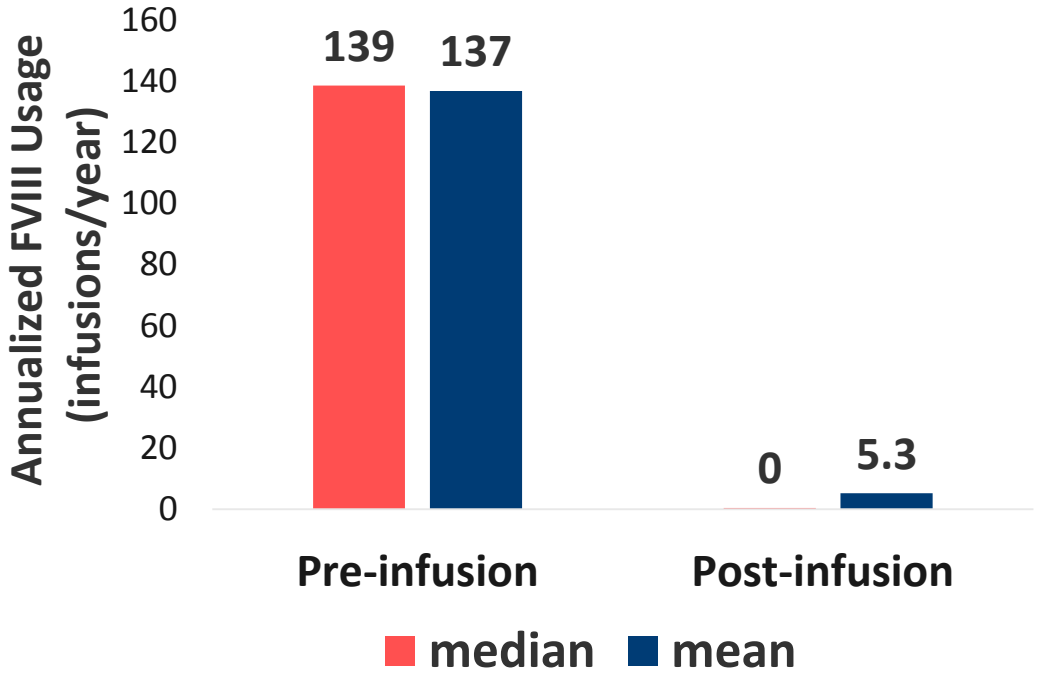
All patients off prophylaxis

SUBSTANTIAL REDUCTION IN MEAN ANNUALIZED FVIII USAGE

STARTING FROM 4 WEEKS POST- INFUSION

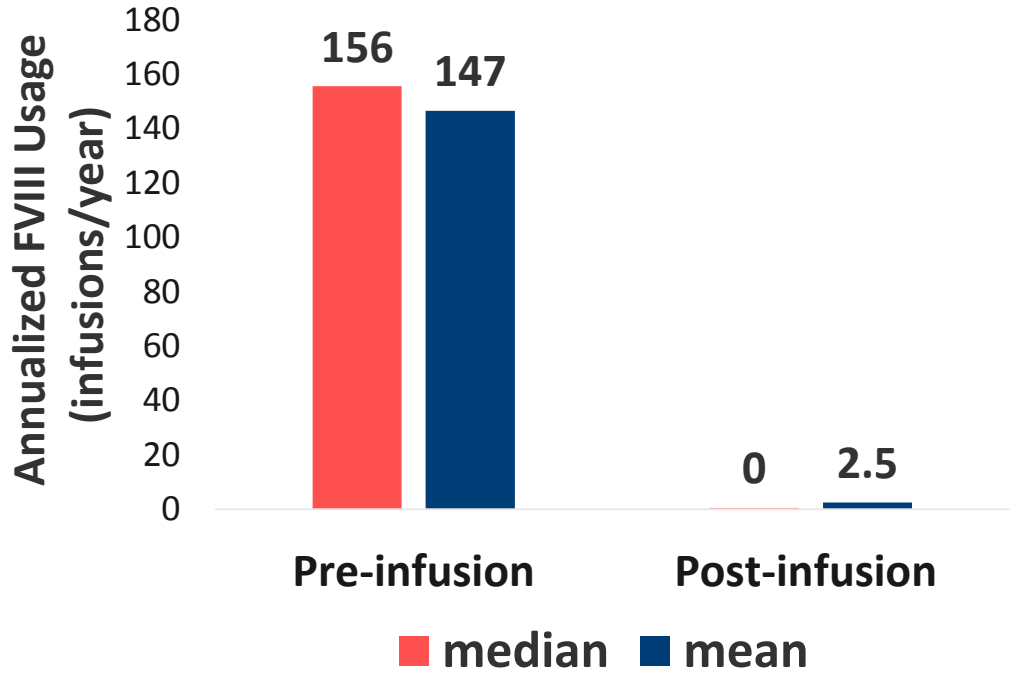
6e13 vg/kg dose through week 104

96% REDUCTION in MEAN FVIII USAGE

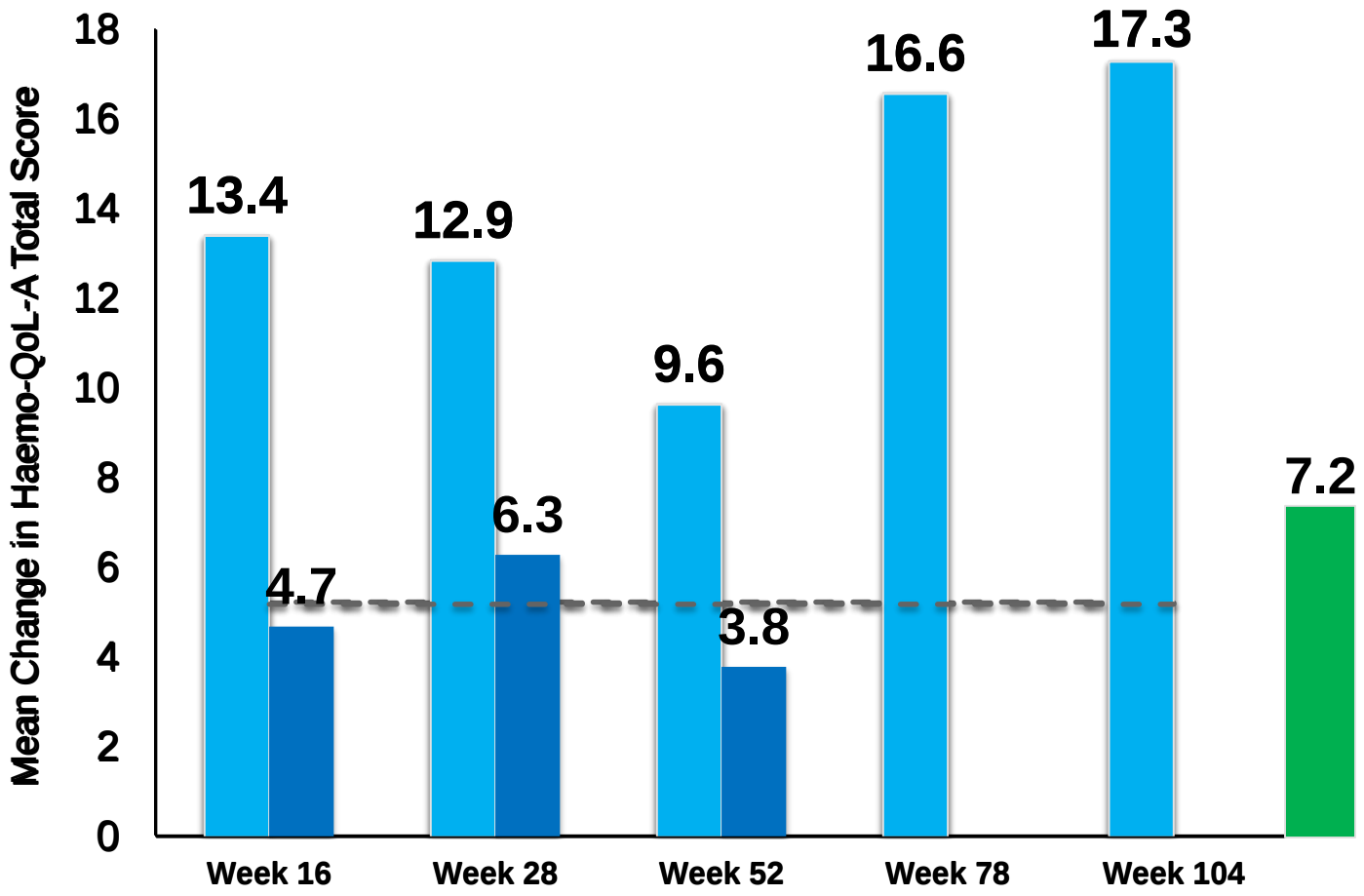


4e13 vg/kg dose through week 52

98% REDUCTION in MEAN FVIII USAGE



BMN 270 SUBSTANTIALLY IMPROVED QOL



QOL improvement observed in all 6 domains; i.e. Consequences of Bleeding, Emotional Impact, Physical Functioning, Role Functioning, Treatment Concern, Worry

6e13 vg/kg cohort

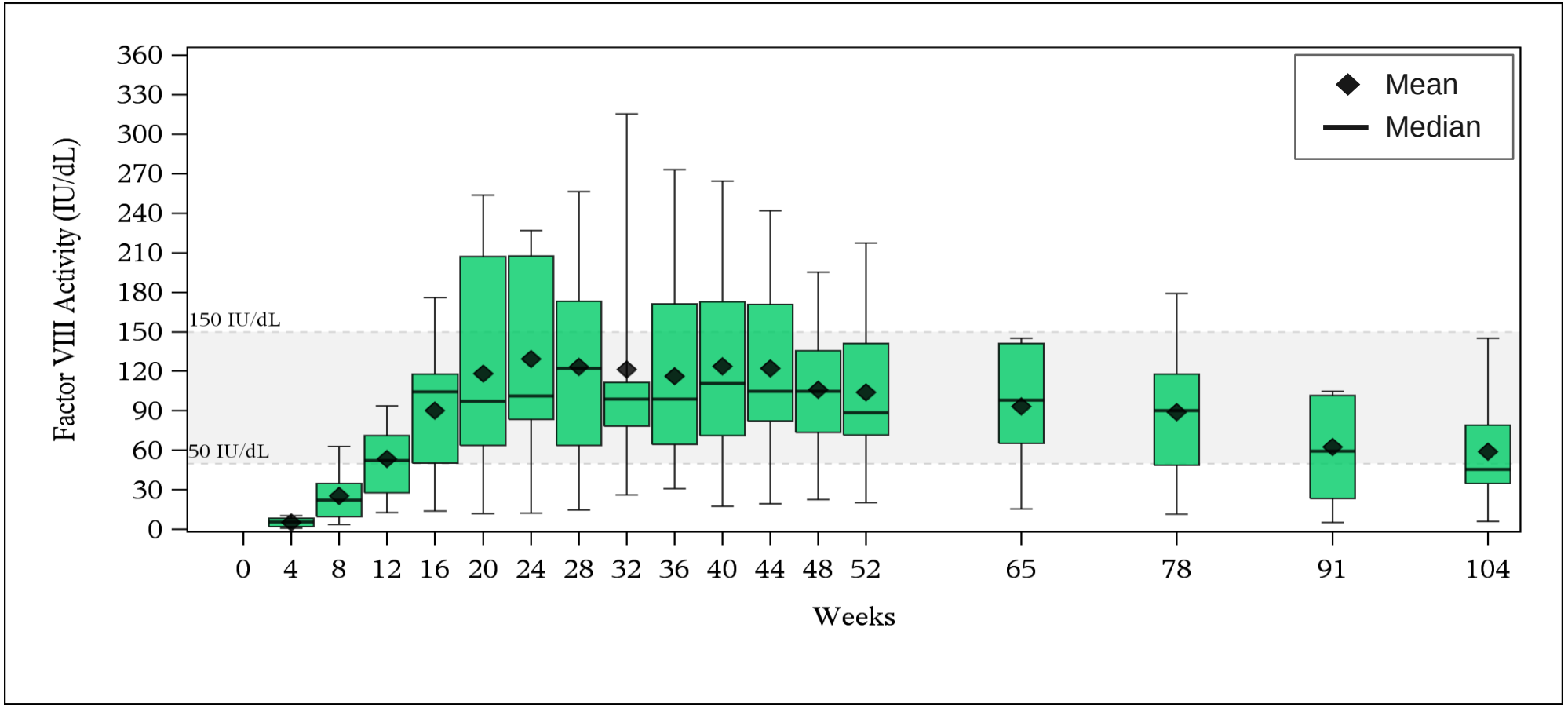
4e13 vg/kg cohort

Spinart result at 3 years*

Dashed line indicate distribution based minimally clinical important difference (MCID) at lower threshold

* Pocoski J et al., 2014

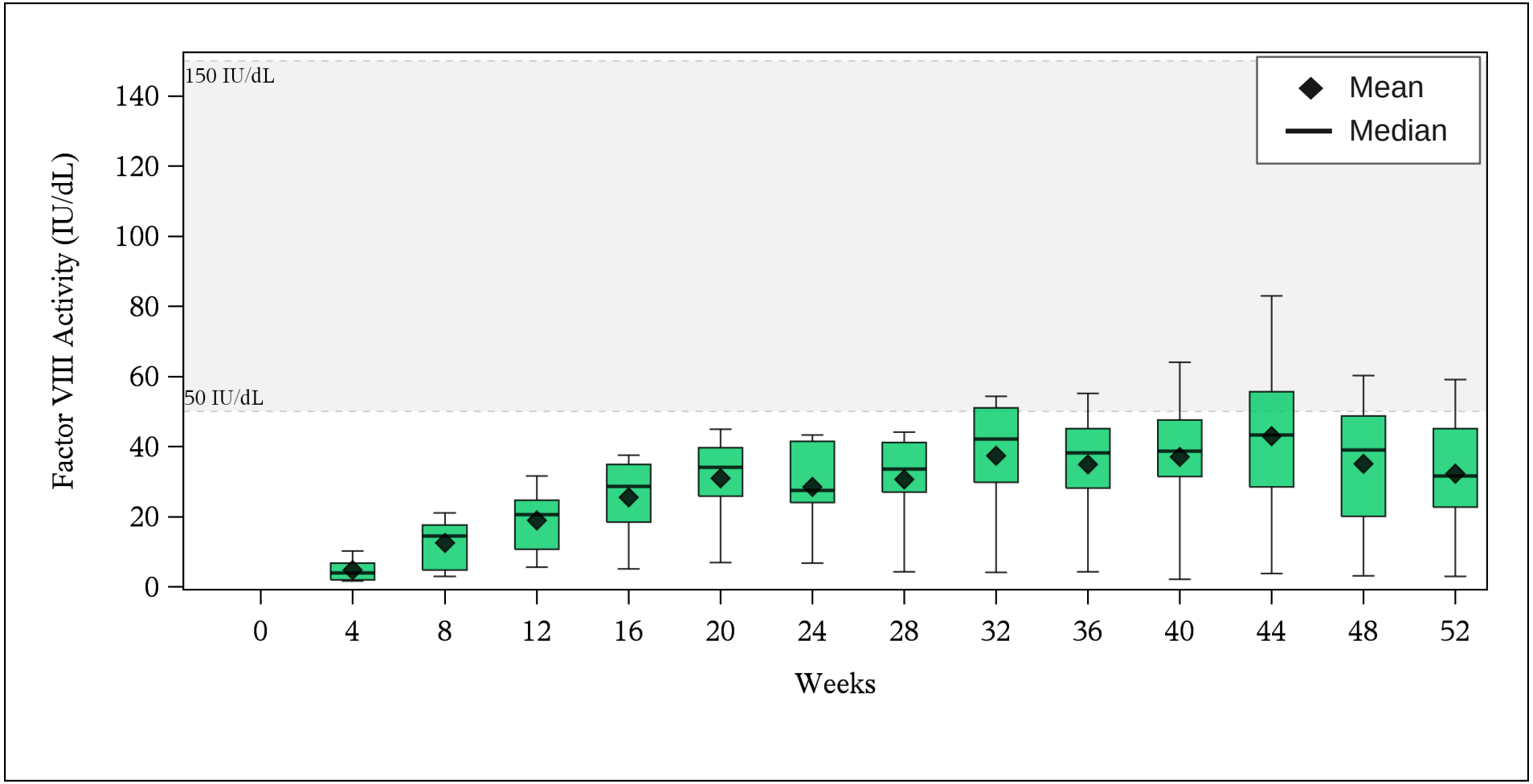
MEAN FVIII ACTIVITY LEVELS SETTLING IN NORMAL RANGE (6e13 VG/KG)



- **No FVIII activity above upper limit of normal at year 2**

The upper and lower box bounds represent 25th and 75th percentiles. The whisker lines represent the minimum and maximum values.

MEAN FVIII ACTIVITY LEVELS AT HIGH END OF MILD RANGE (4e13 VG/KG)



- **No FVIII activity above normal**

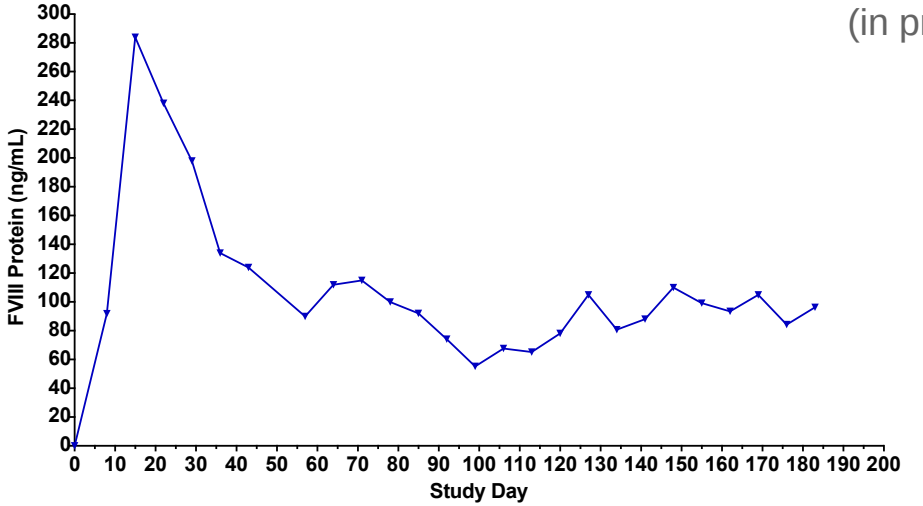
The upper and lower box bounds represent 25th and 75th percentiles. The whisker lines represent the minimum and maximum values.

SUMMARY

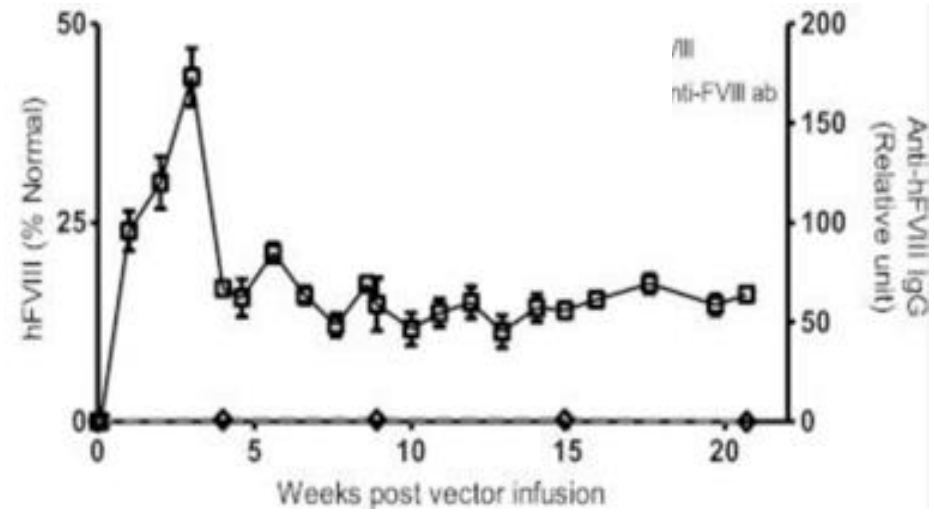
- BMN 270 was well-tolerated with favourable safety profile; currently:
  - No subjects in either cohort have FVIII activity levels above the upper limit of normal
  - ALT levels within normal limits in all subjects
  - All subjects remain off corticosteroids
  - No inhibitors to FVIII
  - Only 2 serious adverse events as previously reported
- ABR: Profound reduction with both cohorts – 6e13 vg/kg sustained 2 years, 4e13 vg/kg sustained 1 year
- FVIII usage: Profound reduction with both 6e13 and 4e13 vg/kg cohorts
- QOL: Continued improvement reflects cessation of bleeding, freedom from worry and independence from treatment
- FVIII activity levels
  - 6e13 vg/kg cohort, settling within normal range
  - 4e13 vg/kg cohort, at upper range of mild haemophilia
- Patterns of FVIII activity levels consistent with other clinical and pre-clinical reports
- Gene therapy has the potential to transform the standard of care in haemophilia A

AAV GENE THERAPY DELIVERS DURABLE EXPRESSION

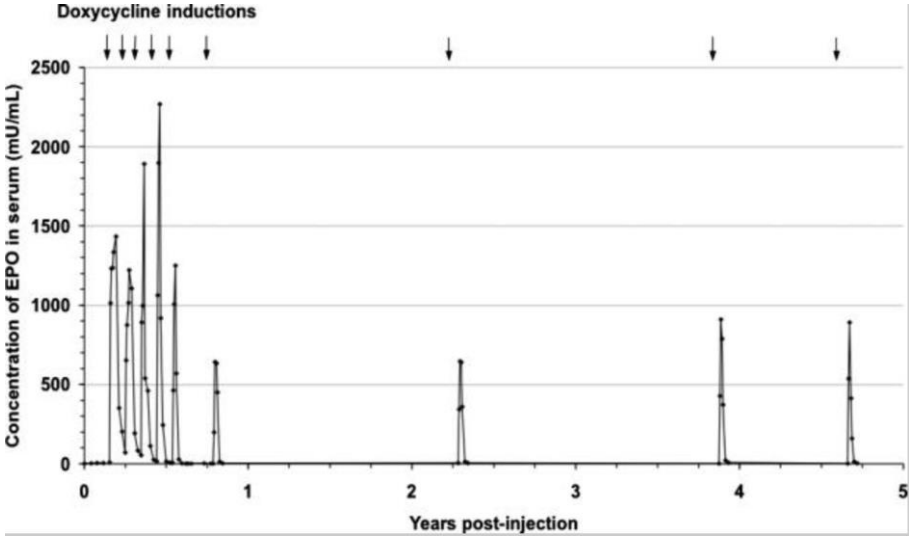
(in pre-clinical models)



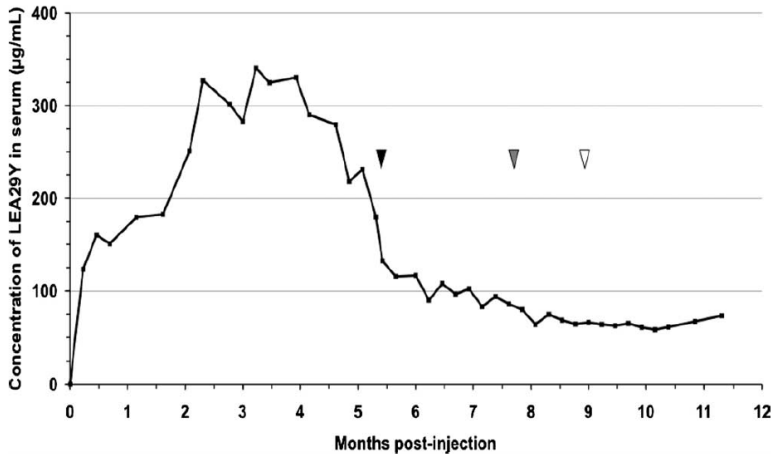
BioMarin data on File



Mcintosh et al Blood 2013



Pernaud-Budloo et al J Virol 2008



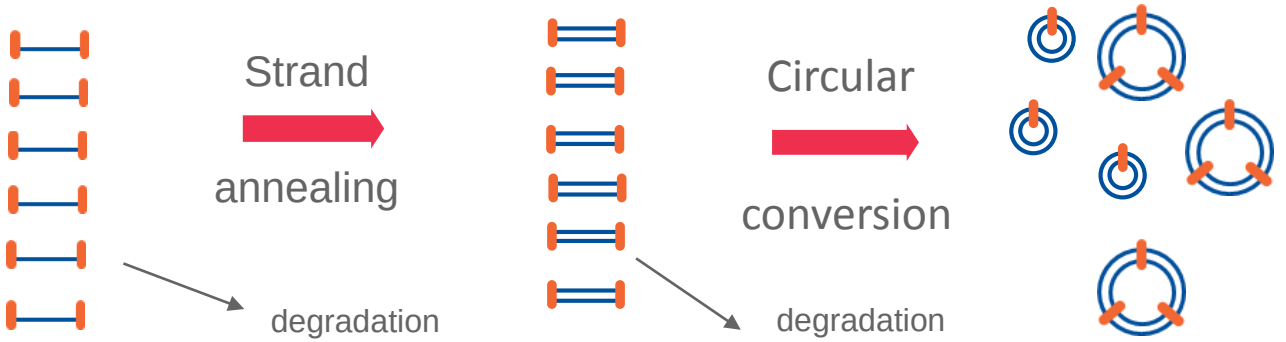
Pernaud-Budloo et al J Virol 2008

HOW AAV VECTORS MEDIATE PERSISTENT EXPRESSION

Incoming SS DNA delivered to nucleus

Expression begins from linear DS genomes

Stable expression persists

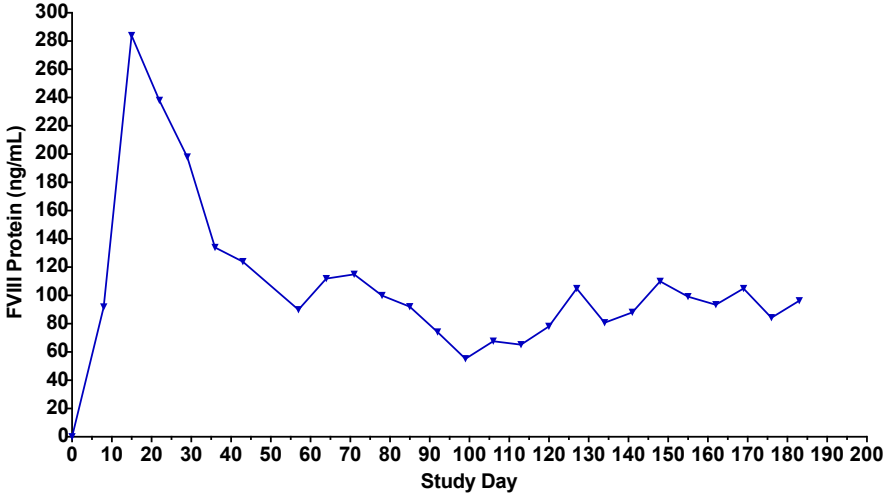


Linear SS-DNA

Linear DS-DNA monomer

Circular DS-DNA monomer and concatamers

The kinetics of expression result from the complex processing of DNA



Lung, liver, muscle of mice & NHP, vector genomes persist as unintegrated, circular episomes.

Afione et al (1996) J Virol 70:3235. Duan et al. (1998) J Virol 72:8568. Nakai et al (2000) J Virol 74:9451. Nakai et al (2001) J Virol 75:6969. Nakai et al (2002) J Virol 76:11343. Nakai et al (2003.)HuGT 14:871. Song et al (2004) PNAS 101:2112. Wang et al (2007) PNAS 104:13104. Snyder et al (1997) HuGT 8:1891. Vincent-Lacaze et al (1999) J Virol 73:1949. Song et al (200) PNAS 98:4084. Penaud-Budloo et al (2008) J Virol 82:7875.

NEXT STEPS

- New goal is to prove superiority of valoctocogene roxaparvovec to prophylactic therapy
- GENEr8-1 (6e13 vg/kg) sample size now powered to evaluate superiority to standard of care
  - 90% powered to demonstrate a reduction in bleeding events
  - N = 130 (90 additional patients)
  - Expect to complete enrollment in Q1 2019
- GENEr8-2 (4e13 vg/kg) study design unchanged
  - N = 40
  - FVIII primary endpoint
  - Targeted to finish enrollment 1-2 quarters after GENEr8-1
- Comprehensive program underway
  - AAV5+ study initiated
  - Global seroprevalance study
  - Ongoing follow-up of 201 patients
  - Initiating use of full commercial scale material from BioMarin manufacturing facility