

# CATALYST BIOSCIENCES

1 & 2 October 2018

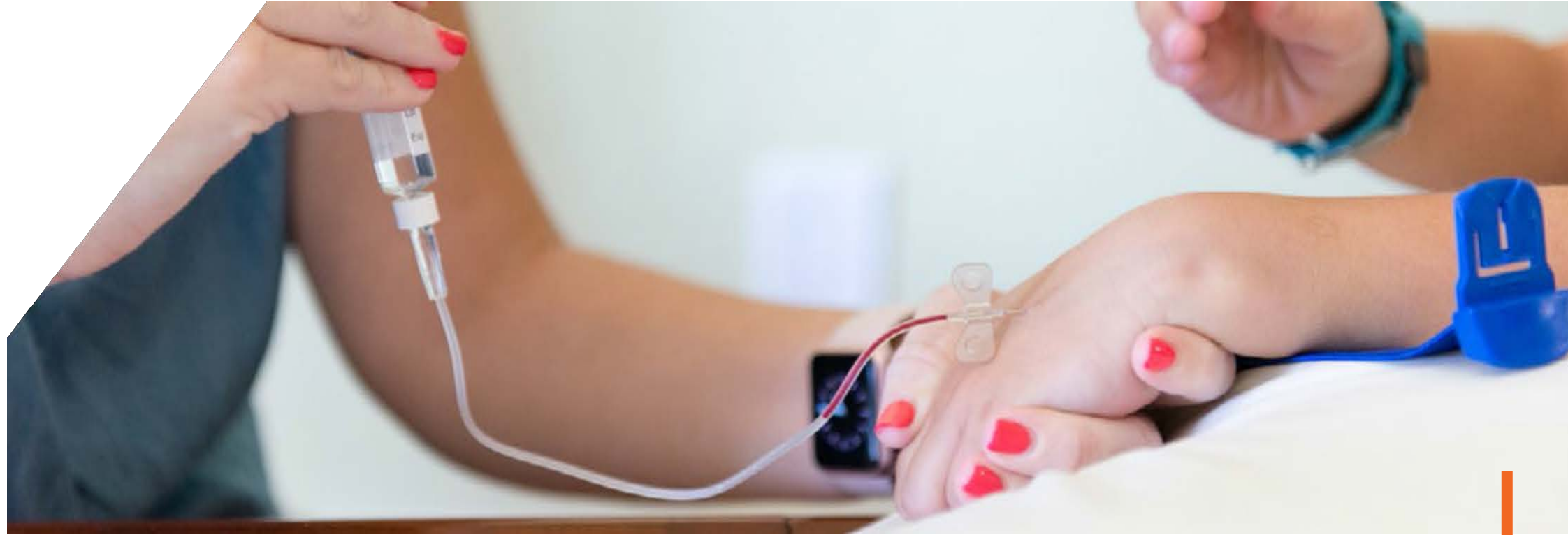
Nassim Usman, Ph.D.  
President & CEO



# Forward looking statements

This presentation includes forward-looking statements that involve substantial risks and uncertainties. All statements, other than statement of historical facts, included in this presentation are forward-looking statements. Examples of such statements include, but are not limited to, the potential benefits of subcutaneous administration of dalcinonacog alfa (formerly CB 2679d/ISU304) and marzeptacog alfa (activated), the potential for long-term dosing of dalcinonacog alfa to maintain FIX activity in the high-mild hemophilia range, statements relating to Catalyst's clinical trial timelines, including plans to complete patient enrollment of the Phase 2/3 trial of marzeptacog alfa (activated) by the end of 2018 and plans to announce data during 2018 or file an IND for acquired hemophilia in 2019, plans for the initiation of a Phase 2b clinical trial of dalcinonacog alfa in the first quarter of 2019, and the potential market opportunities for these products. Actual results or events could differ materially from the plans and expectations and projections disclosed in these forward-looking statements. Various important factors could cause actual results or events to differ materially from the

forward-looking statements that Catalyst makes, including, but not limited to, the risk that trial initiation or enrollment may be delayed and that ongoing or future trials may not achieve their endpoints, that subsequent clinical trials will not replicate the results from earlier clinical studies on small numbers of patients, that potential adverse effects may arise from the testing or use of Catalyst's products, including the generation of antibodies or inhibitors, the risk that costs required to develop or manufacture Catalyst's products will be higher than anticipated, the risk of competition from other hemophilia treatments, including those in development, Catalyst's ability not to infringe third party intellectual property rights, and other factors described in the "Risk Factors" section of Catalyst's Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, which was filed with the Securities and Exchange Commission on August 2, 2018. Forward looking statements in this presentation speak only as of the date hereof. Catalyst does not assume any obligation to update any forward-looking statements, except as required by law.



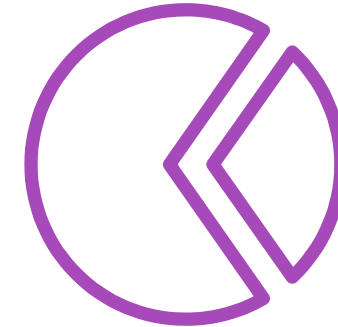
We are working to establish a **new** standard of care in **hemophilia prophylaxis** by developing highly potent **subcutaneous treatments** that improve the quality of life for patients with hemophilia with inhibitors, acquired hemophilia & hemophilia B



# Investment highlights



Novel subcutaneous compounds with orphan drug designation



Market: \$3.4B in annual sales



FVIIa & FIX SQ efficacy clinically demonstrated

2018

FVIIa Phase 2 top-line data expected in Q4 2018



Experienced team



~134 worldwide patents – CBIO retains full ownership of all compounds

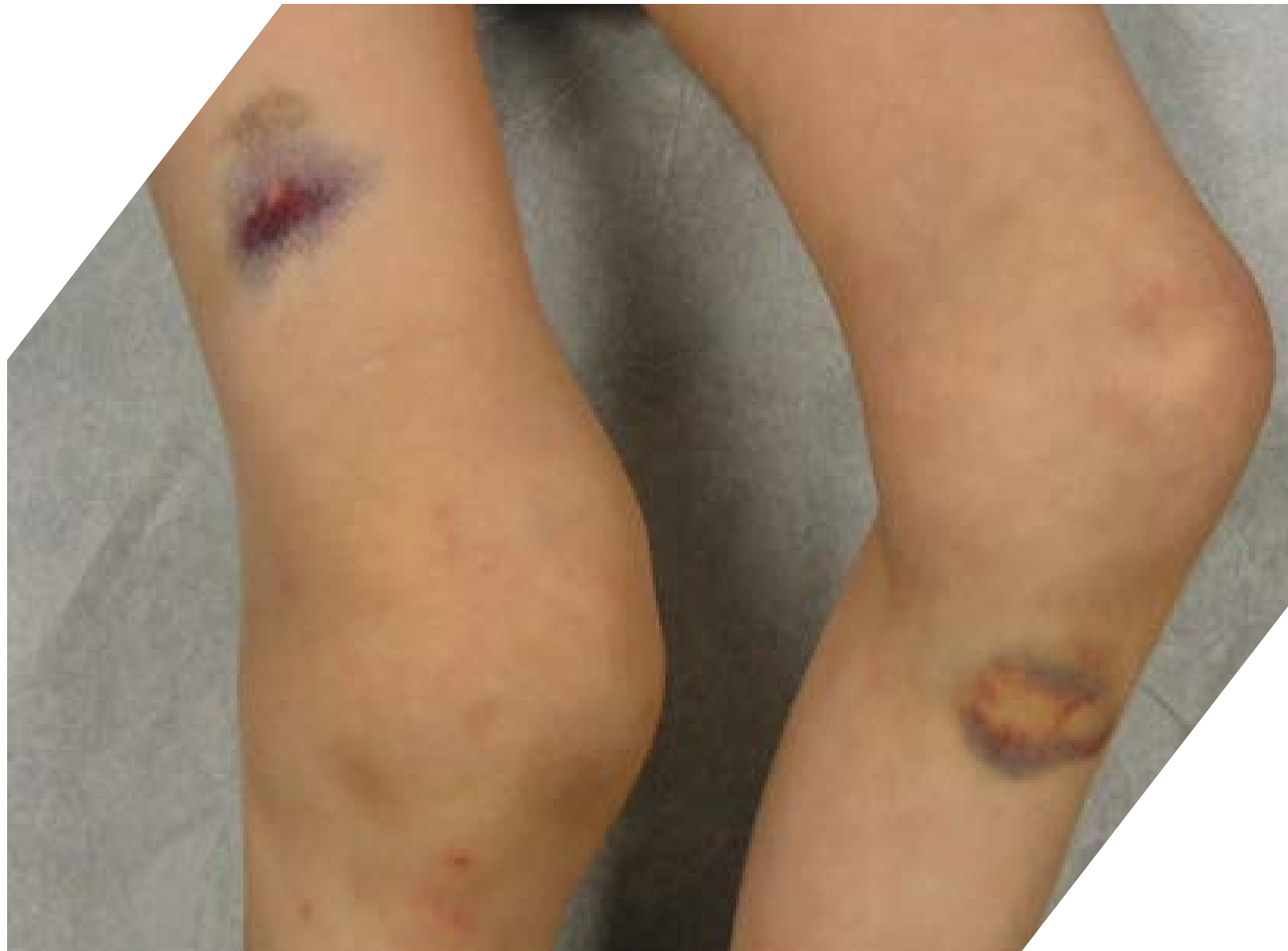


Well funded

\$136M cash (Q2 2018)



# Life with hemophilia



## Hemophilia with inhibitors

- A complication in factor replacement therapy that neutralizes the treatment
- 30% of Hem A (FVIII) patients and 5% of Hem B (FIX) patients develop inhibitors
- Patients are at high risk for hemorrhagic stroke and premature mortality

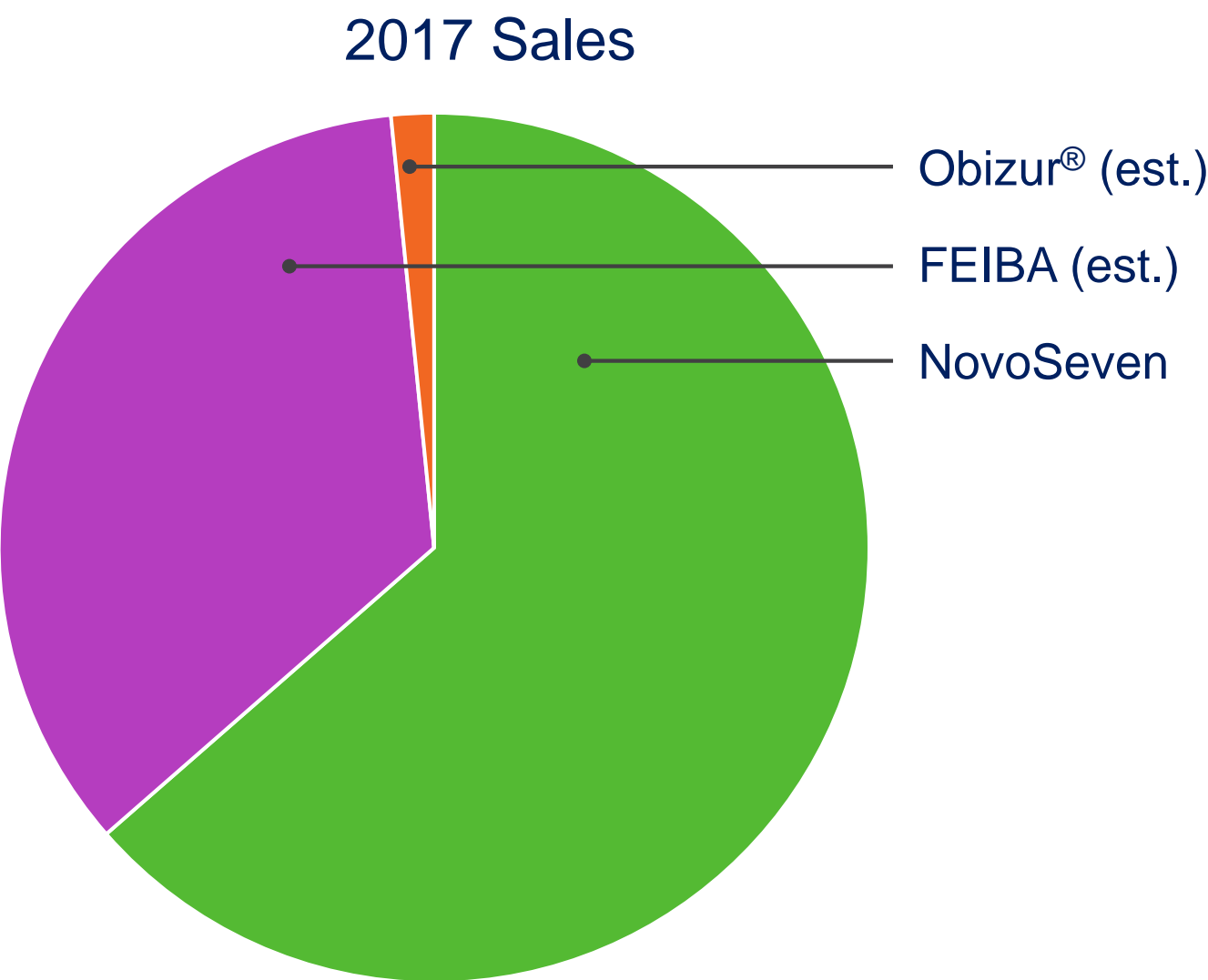
## Hemophilia B

- Rare disorder, FIX deficient, mostly inherited but can be caused by a spontaneous mutation
- Causes spontaneous bleeding, mostly into joints, resulting in disabling joint damage

## Acquired Hemophilia

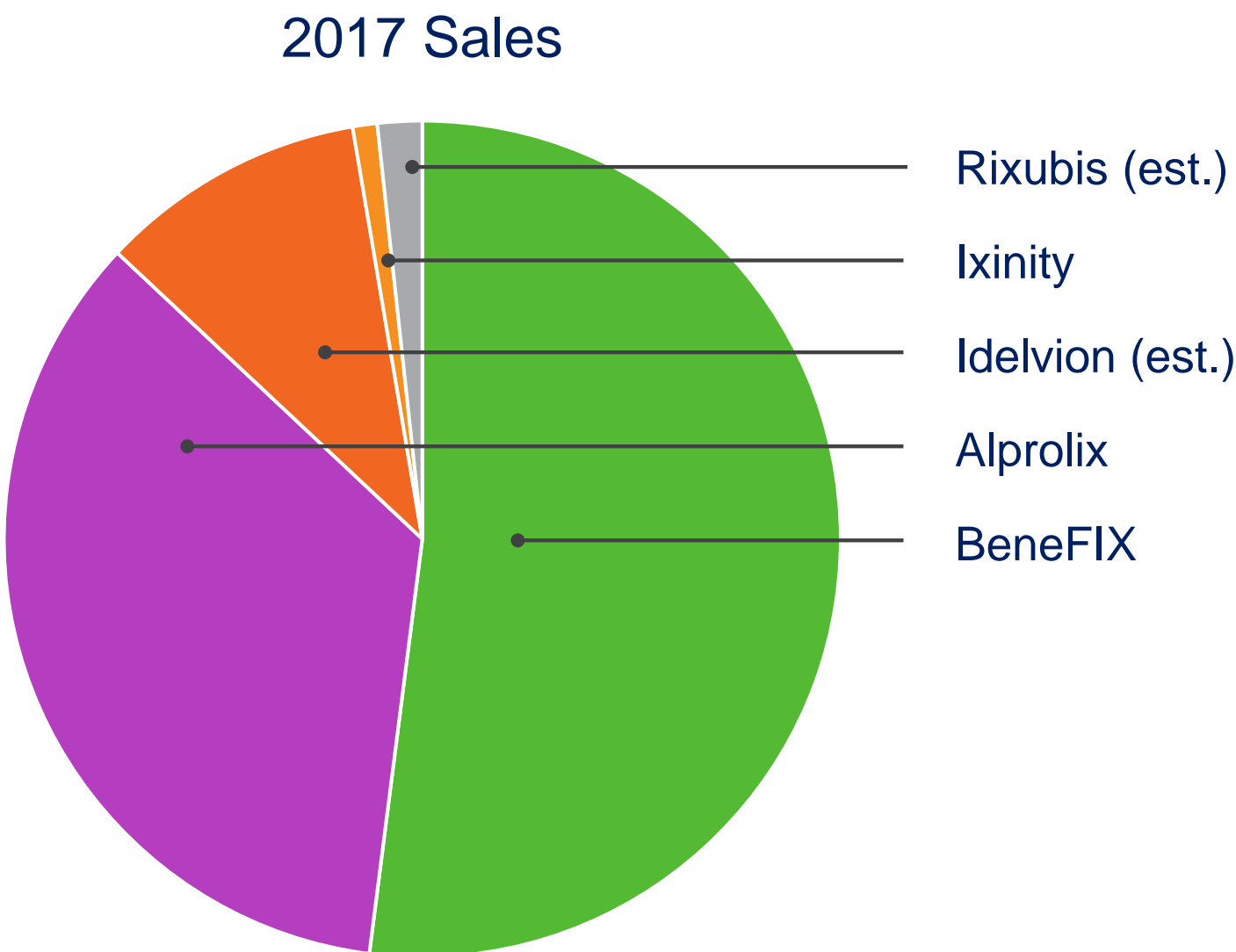
- Rare disorder, occurs spontaneously, bleeding caused by anti-FVIII nAbs
- Currently treated with immunosuppressants + IV bypass agents (FVIIa, FEIBA<sup>®</sup> or Obizur<sup>®</sup>)
- Unmet need to adequately treat and prevent re-bleeds

FVIIa & Bypassing Agents: \$2.2B market



In 2017 over 2,400 US and EU5 patients were treated with FVIIa and bypassing agents for hemophilia with inhibitors, acquired hemophilia and factor VII deficiency

Hemophilia B, FIX: \$1.2B market



In 2017 over 6,000 US and EU5 hemophilia B patients were treated with recombinant FIX

Sources: WFH Annual Global Survey, GlobalData, Roche, Novo Nordisk, Aptevo, SOBI, Bioverativ. \*Hemlibra had global sales of \$58M in 1H 2018

# Available treatments



- Regular intravenous (IV) infusions are necessary to maintain higher clotting levels
- IV treatments are very unpleasant and time-consuming
- Inconvenience affects compliance, outcomes and quality of life
- Especially difficult for pediatric patients & their families





# The Catalyst Biosciences solution



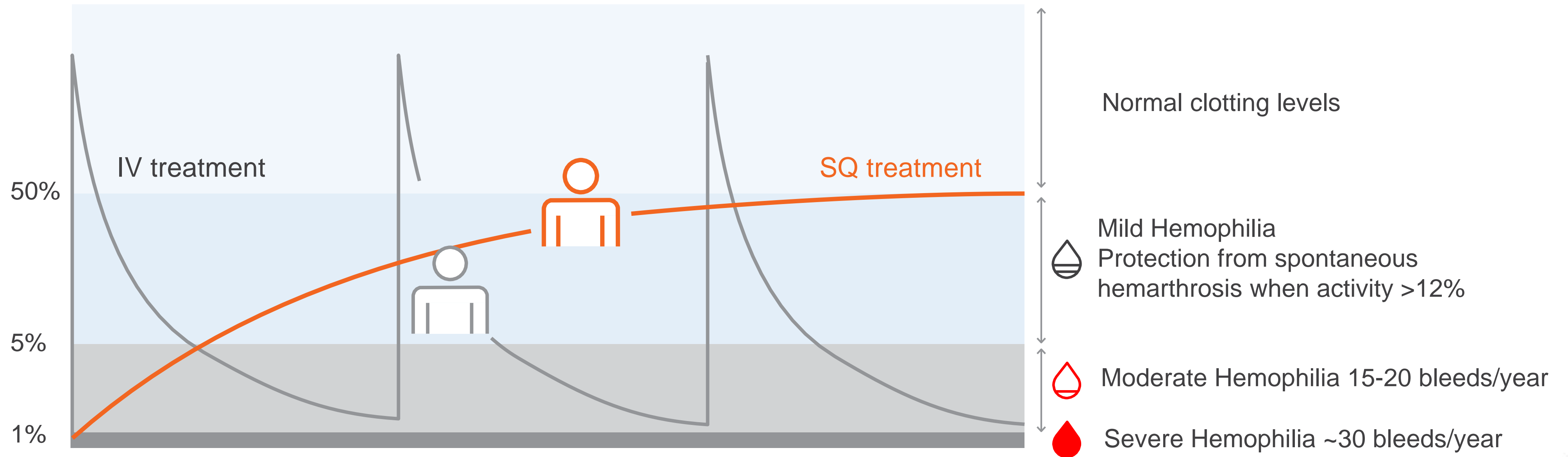
## Our highly potent solution:

- + Quick & simple subcutaneous injection – allows for self-administration including in pediatric patients
- + Much higher & stable factor levels – keeps patients at safe levels for much longer



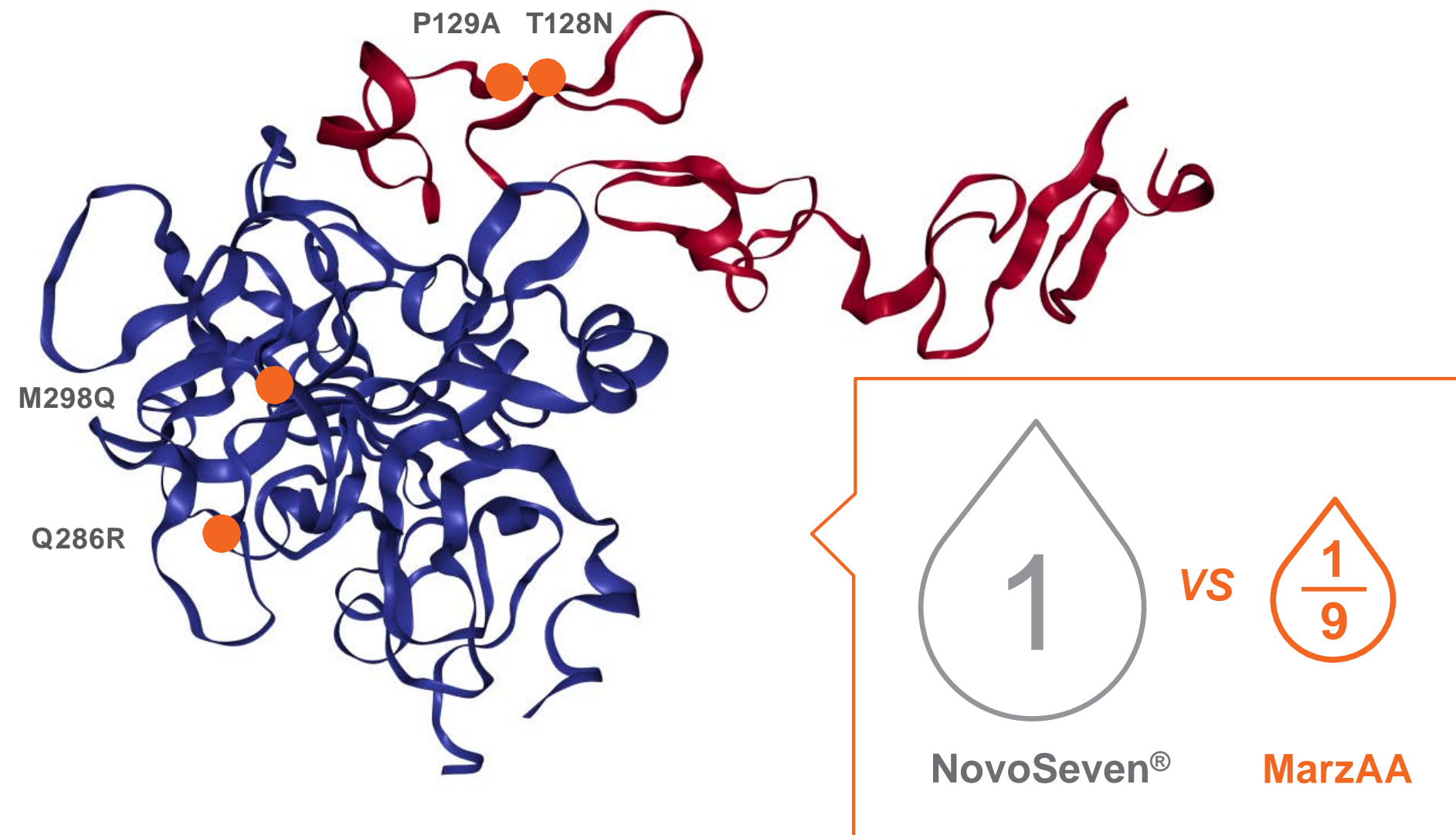
# The new standard in hemophilia prophylaxis

**Patients in high mild range are protected from spontaneous bleeds**



- + The concept of prophylactic treatment is to keep severe & moderate hemophilia patients in the high mild range
- + Our subcutaneous treatment has the ability to build up over time, offering long-term stability in clotting levels

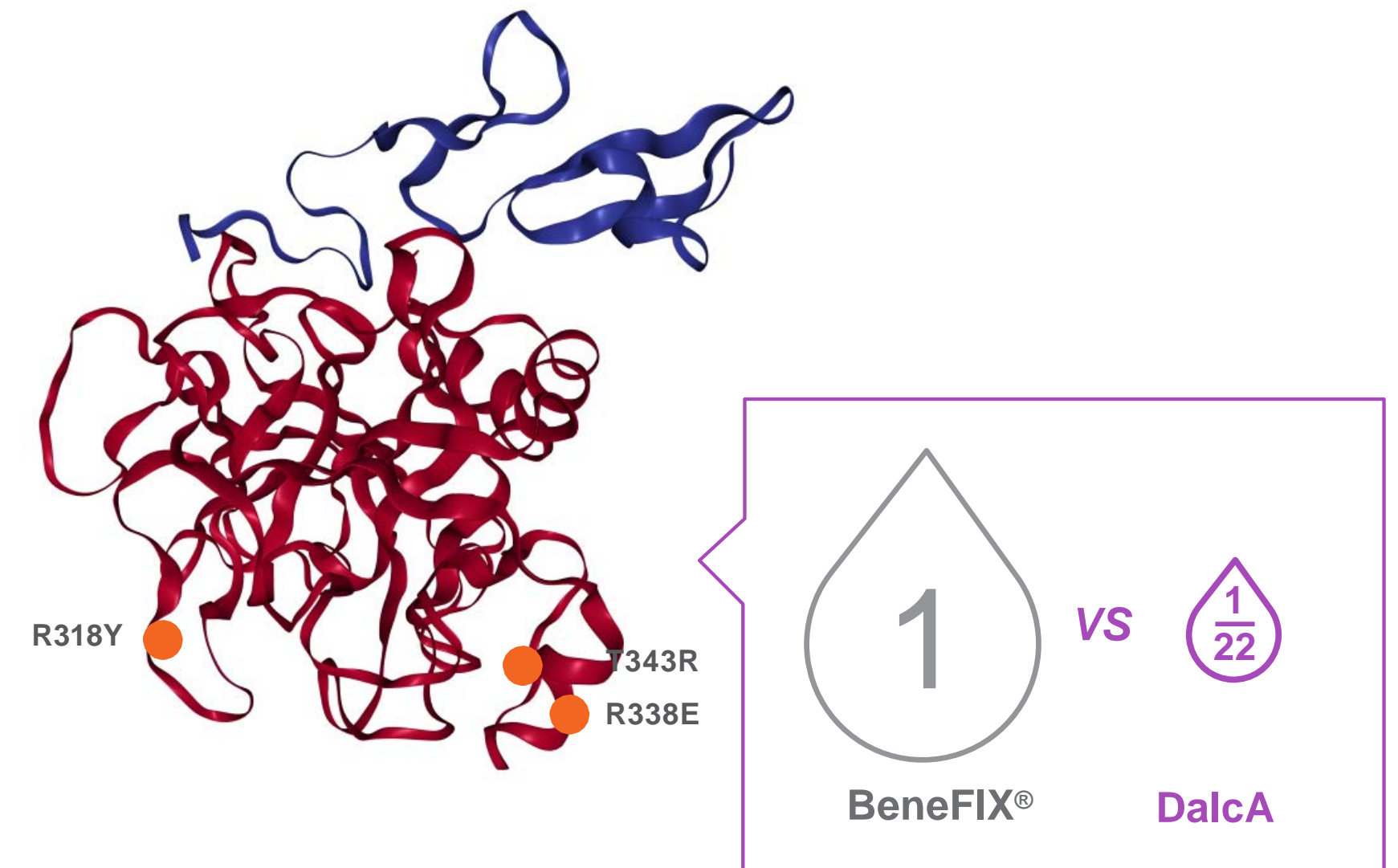
# Our products



## Factor VIIa Marzeptacog alfa (activated) – MarzAA

Hemophilia with inhibitors & acquired hemophilia

- + 9-fold more potent than NovoSeven®
- + Allows SQ injection
- + Worldwide patents through 2029
- + US orphan drug designation



## Factor IX Dalcinonacog alfa – DalcA

Hemophilia B

- + 22-fold more potent than BeneFIX®
- + Allows SQ injection
- + Worldwide patents through 2031
- + US & EU orphan drug designation

# Pipeline





# MarzAA phase 1 IV clinical trial results\*

## Hemophilia with inhibitors FVIIa

- ✓ 9-fold potency advantage vs NovoSeven
- ✓ 25 severe hemophilia patients with and without inhibitors
- ✓ Demonstrated pharmacological efficacy by significant shortening of aPTT (activated partial thromboplastin time) and PT (prothrombin time)
- ✓ No inhibitors or thrombosis

“MarzAA would conservatively capture >10% hemophilia A inhibitor patients, not every patient will go on, or stay on ACE910”

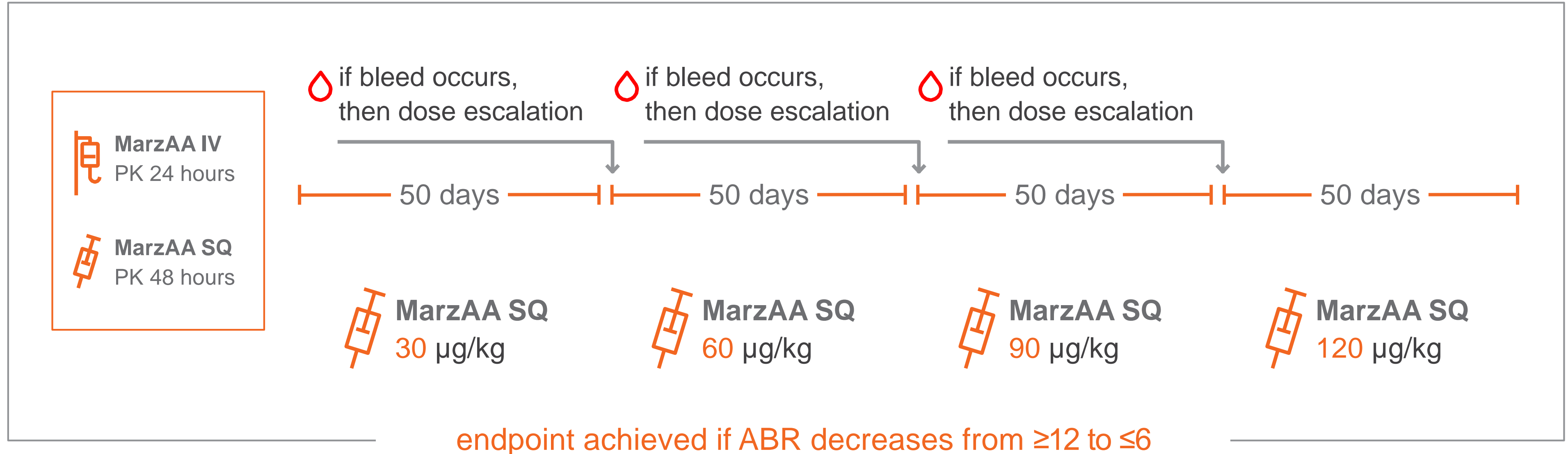
“Severe FVII deficient patients would want to switch to MarzAA... a daily SQ could ‘normalize’ them”

“There is a clear unmet need for a SQ therapy in acquired hemophilia and MarzAA could fill that need, I think it is an excellent idea”

“MarzAA would become 1st line treatment for all hemophilia B inhibitor patients”

# MarzAA phase 2 SQ clinical trial design

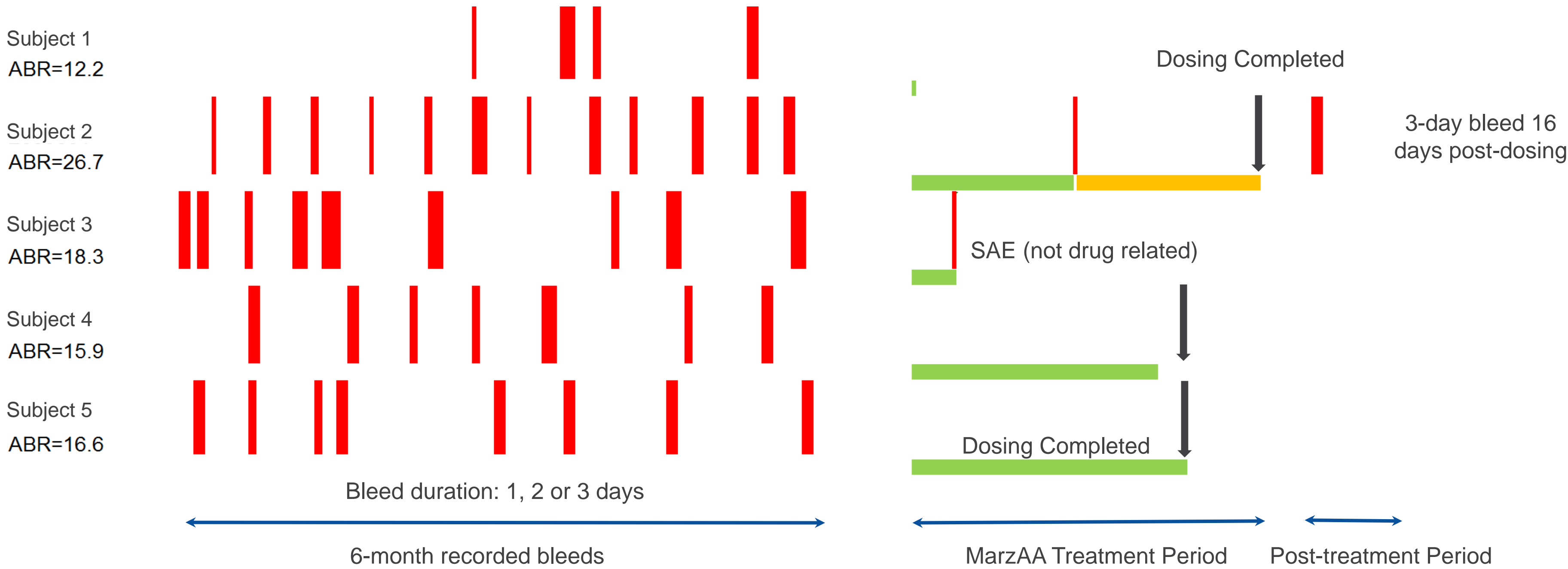
## Hemophilia with inhibitors: FVIIa



- + Open label SQ study with individual dose escalation if needed
- + Hemophilia A or B with inhibitors
- + Up to 12 adult patients with documented annual bleeding rate (ABR)  $\geq 12$
- + Primary endpoint: safety and tolerability
- + Secondary endpoints: reduction in annual bleed rate, no inhibitor formation

# MarzAA reduces annualized bleed rate (ABR)

MarzAA 30  $\mu\text{g/kg}$  & 60  $\mu\text{g/kg}$



Anticipate completing enrollment of up to 12 subjects by end of 2018 – interim data at ASH 2018



# Dalcinonacog program summary

## Hemophilia B FIX

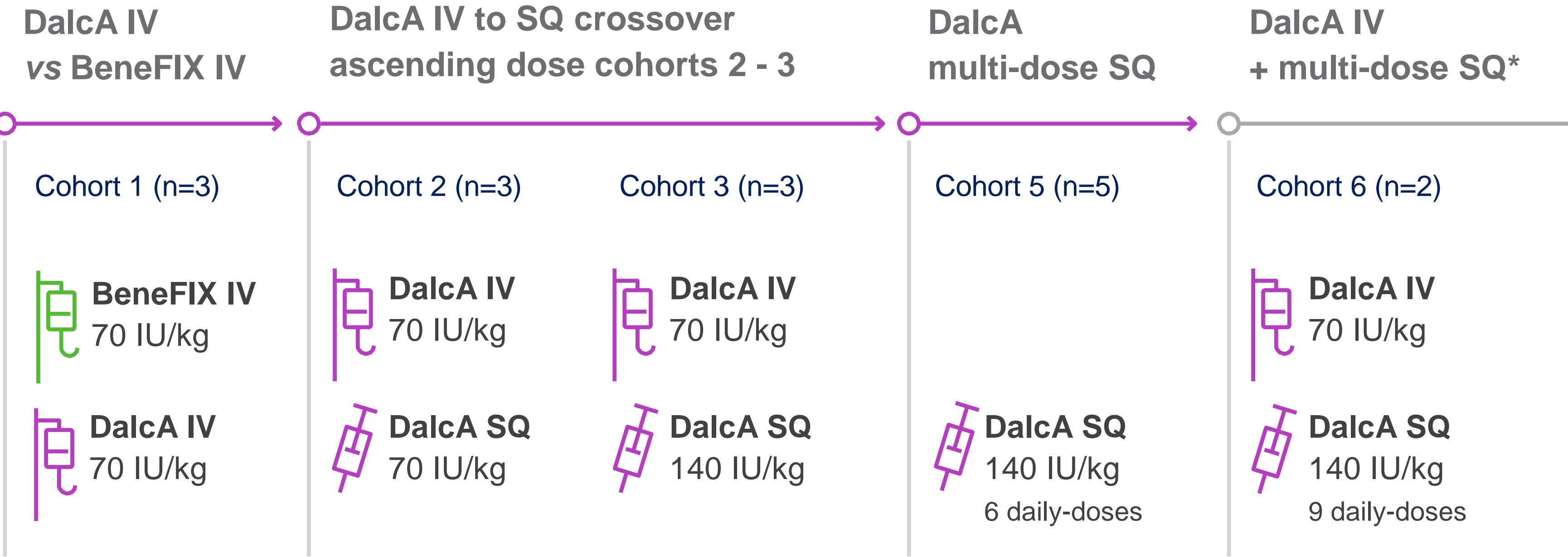
- ✓ 22-fold potency advantage vs BeneFIX allows subcutaneous administration
- ✓ Only 6 daily SQ doses (140 IU/kg) needed to correct severe hemophilia to mild, **15.7% median FIX activity**
- ✓ IV loading dose (70 IU/kg) followed by daily SQ dosing (140 IU/kg) for 9 days resulted in **>30% FIX activity**
  - nAbs detected, one transient
  - Does not cross react with wt-FIX
  - Analysis ongoing
- ✓ Phase 2b to explore longer-term dosing pending outcome of nAb analysis

**“These exciting results demonstrate for the first time the feasibility of a subcutaneous FIX injection to provide meaningful protection from bleeding, even after only six doses”**

**Dr John Pasi,**  
Professor of Haemostasis & Thrombosis  
at Barts and The London School of Medicine

# Dalcinonacog phase 1/2 open label design

## Hemophilia B FIX

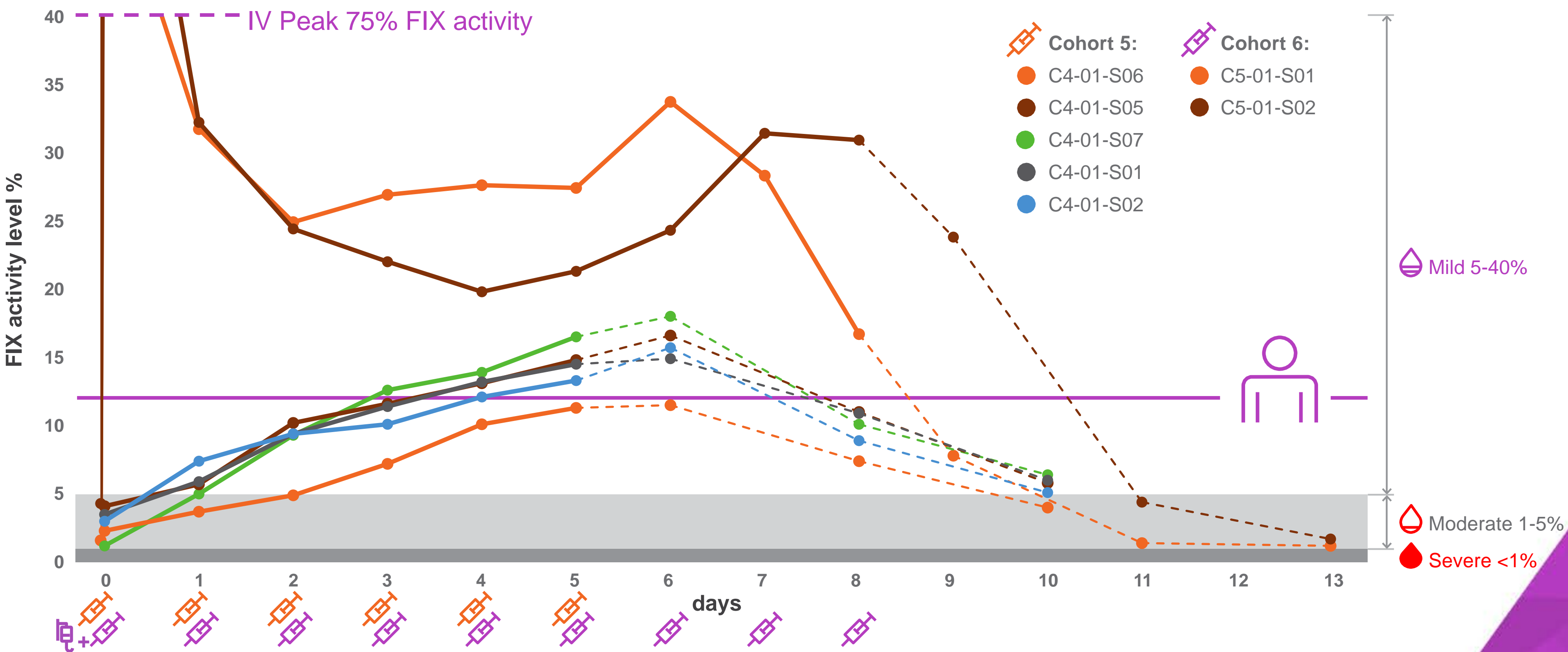


\*First SQ dose 30 min post-IV

# Phase 1/2: Cohort 5 & 6 FIX activity results

## Hemophilia B FIX

6/7 patients had trough levels >12%, sufficient to protect against spontaneous joint bleeds





# DalcA nAb analysis & outcomes – Q4 2018

## Investigation

HLA Typing / Immunogenicity  
(Certain HLA types increase risk of ADAs)

DP Quality Characterization  
(Drug quality induces ADAs)

DP Formulation Characterization  
(Formulation induces ADAs)

*In Silico & In vitro* Immunogenicity  
(Molecule is inherently immunogenic)

## Potential Outcomes

Restrict HLA

Continue with DalcA & current formulation

Manufacture new batch & formulation

Move to Back-Up Compound

# CB 2782 anti-C3 protease

## Dry AMD



- + Dry AMD is an advanced form of age-related macular degeneration that results in the irreversible loss of light-detecting cells and leads to blindness
- + Global market is >\$5B with no approved drugs
- + C3 in the complement cascade is a clinically validated target for the treatment of Dry AMD

# CB 2782 anti-C3 protease summary

## Dry AMD

### CB 2782 intravitreal injection



### APL-2 intravitreal injections



- + Prevent the progression of geographic atrophy
- + Our novel anti-C3 protease is derived from a human protease and completely inhibits C3 in non-human primate studies
- + Catalytic degradation of C3 may allow for ~once quarterly intravitreal dosing, compared with monthly dosing of leading competitor
- + Preclinical data with longer acting proteases expected in 2018



# Team

## President & CEO

Nassim Usman, Ph.D.

## SVP, Technical Operations

Andrew Hetherington, M.B.A.









## Chief Medical Officer

Howard Levy, M.B.B.Ch., Ph.D., M.M.M.

## VP, Translational Research

Grant Blouse, Ph.D.













## Chief Financial Officer

Fletcher Payne

## VP, Business Development

Jeffrey Landau, M.B.A.













# Milestones

	2018				2019		
	Q1	Q2	Q3	Q4	Q1	Q2	Q3
<div>Marzeptacog alfa</div> <div>(activated)</div> <div>(FVIIa)</div>	<div>P2 Initiated</div> <div>✓</div>		<div>ISTH</div> <div>Interim P2 data</div> <div>✓</div>	<div>ASH</div> <div>P2 data</div>	<div>EAHAD</div> <div>P2 data</div>	<div>Hemophilia with Inhibitors</div> <div>EoP2</div> <div>Acquired Hemophilia</div> <div>IND</div>	
<div>Dalcinonacog alfa</div> <div>(FIX)</div>	<div>EAHAD</div> <div>Top-line multidose clinical data (oral)</div> <div>✓</div>	<div>WFH</div> <div>Final Cohort 5 data</div> <div>Initiate Cohort 6</div> <div>✓</div>	<div>ISTH</div> <div>Phase 1/2 Cohort 6 data</div> <div>✓</div>		<div>Initiate P2b</div> <div>EAHAD</div>		<div>ISTH</div> <div>P2b data</div>

## Operating Results

### 2Q/2018

### Year-to-Date

Operating Expense .....	\$7.1 M	\$13.8 M
Net Loss .....	(\$6.5 M)	(\$11.5 M)
Net Loss per share .....	(\$0.54)	(\$1.10)

## Share Data

Common Stock Outstanding.....	11,942,729
Fully Diluted Shares.....	14,623,688
Average Volume.....	843,064
Market Capitalization as of 28 September 2018.....	~\$128 M

## Financial Strength

Cash & Cash Equivalents Q2/2018.....	\$136.1 M
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CBIO stock is included in the Russell 3000 index

+ Funds increasing their position in CBIO: BlackRock, Vanguard, State Street Global, Geode Capital, Northern Trust



# Summary

## ✓ **Disruptive approach to a \$3.5 billion market**

Subcutaneous prophylactic dosing designed to be less painful and much more convenient, especially for children

+ Clinical proof of efficacy demonstrated for both Marzeptacog alfa (activated) & Dalcinonacog alfa

## ✓ **FVIIa: Marzeptacog alfa (activated)** **~\$2.2 Billion market**

Phase 2 of a Phase 2/3 program enrolling

90% reduction in ABR on treatment

No ADAs or nAbs observed to date

+ Phase 2 data at ASH 2018

+ EoP2 & IND for Acquired Hemophilia in Q2 2019

## ✓ **FIX: Dalcinonacog alfa** **~\$1.2 billion market**

>30% activity levels achieved with daily SQ dosing

Potential to maintain long-term FIX activity in the mild hemophilia range to be explored in P2b

+ Initiate Phase 2b in Q1 2019 pending nAb analysis

## ✓ **Anti-C3 for Dry AMD:** **multi-billion market opportunity**

C3 is a clinically validated target, potential to generate a best-in-class molecule

+ Pre-clinical proof-of-concept in 2018

## ✓ **Cash runway allows independent development of lead programs**

# THANK YOU

Nasdaq: CBIO  
catalystbiosciences.com

design by  
**THEORIA**  
CREATIVE