BCX9930 Data Update

September 30, 2020

Forward-Looking Statements

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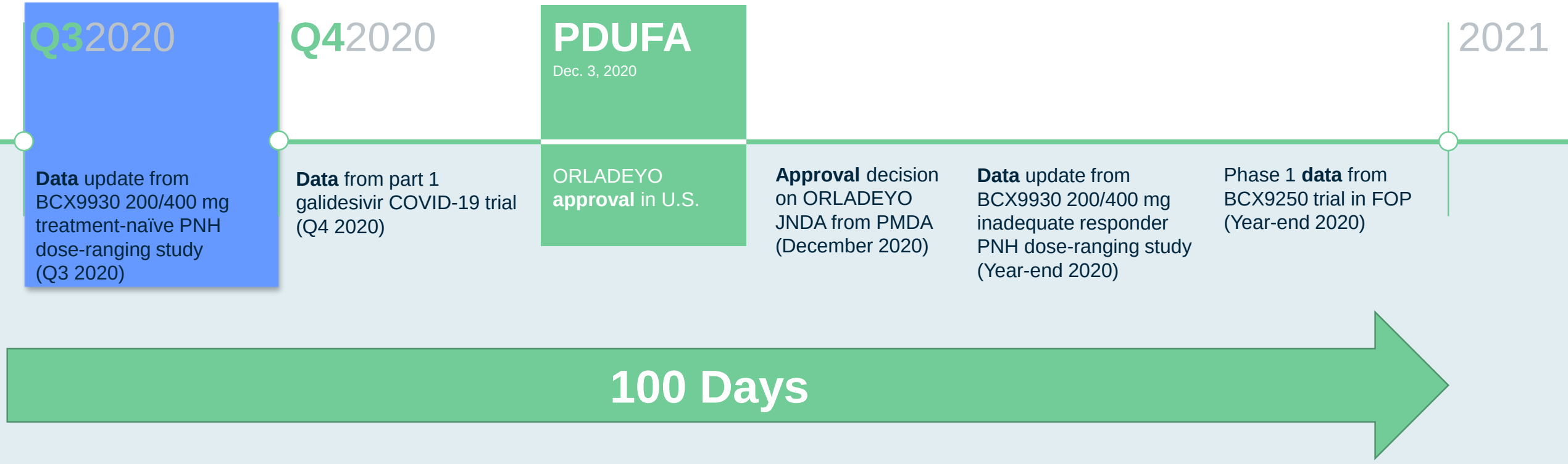
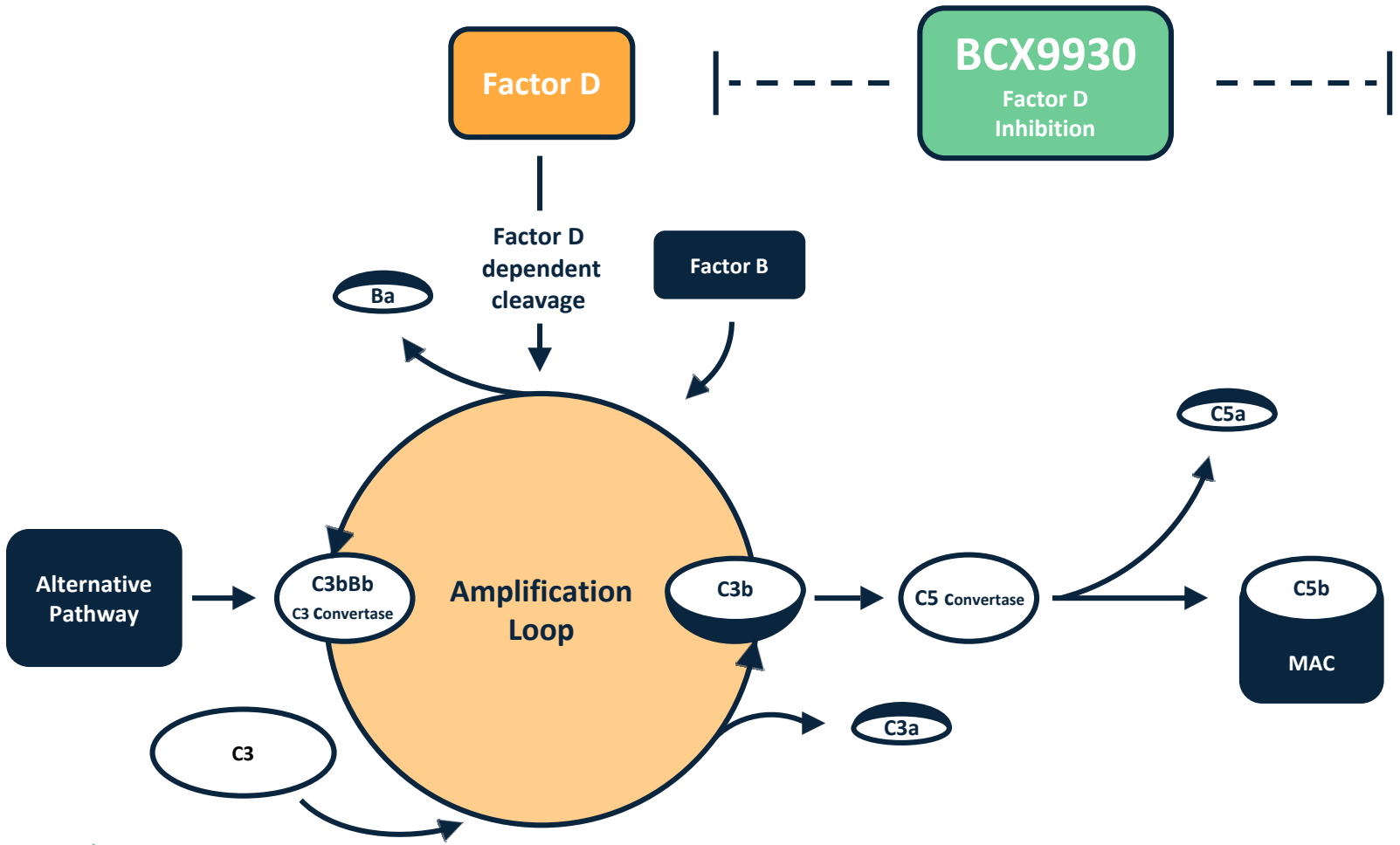
Six Major Program Updates Expected in Next 100 Days



Factor D: An Outstanding Drug Target for Complement-mediated Diseases

- Factor D is essential to initiate the Alternative Pathway
- Blocking Factor D blocks the Alternative Pathway and all downstream products



Spectrum of Alternative Pathway Dysregulation Diseases

Hematology

PNH
paroxysmal nocturnal hemoglobinuria

aHUS
atypical hemolytic uremic syndrome

Rheumatology

ANCA vasculitis
antineutrophil cytoplasmic antibody-associated vasculitis

Lupus nephritis

IgAN vasculitis

Nephrology

C3G
glomerulonephritis

PMN
primary membranous nephropathy

IgAN
IgA nephropathy

Oral Monotherapy with BCX9930 Offers Advantage of Controlling Both Intravascular (IVH) and Extravascular (EVH) Hemolysis

Goal of Total Hemolytic Control

IVH Control

↓ LDH

+

EVH Control

↓ Opsonized RBCs

Hemolytic Control

↑ RBC/Granulocyte Clone Size

Patient Outcomes from Controlled Hemolysis over Time

Relieve Anemia

↑ Hemoglobin

Reduce PNH Clinical Symptoms

↓ Appearance of Symptoms

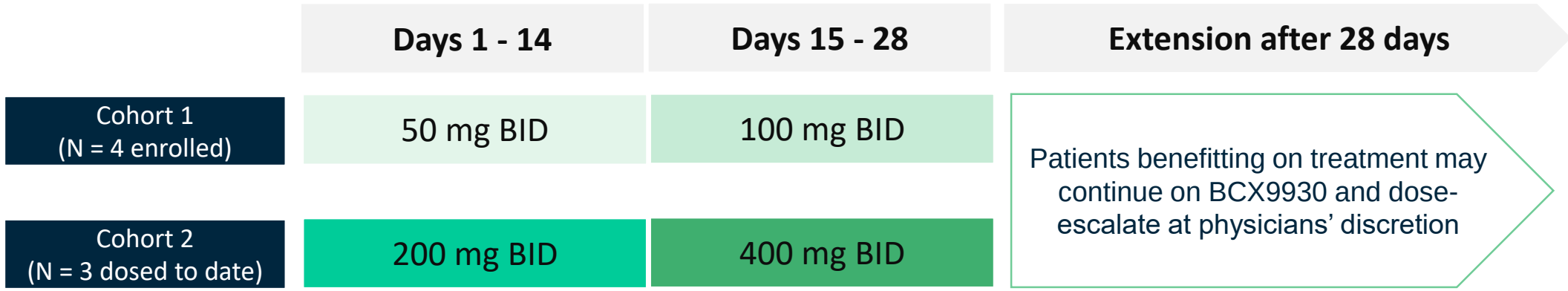
Avoid Transfusions

↓ Transfusions

A. M. Risitano *et al.*, *Front Immunol* **10**, 1157 (2019)

PNH Proof of Concept Study Design: BCX9930 as Monotherapy

BCX9930 Study Design: Patients with PNH who are Naïve to C5-INH Treatments



Key Eligibility Criteria at Screening: Patients with PNH who are Naïve to C5-INH Treatments

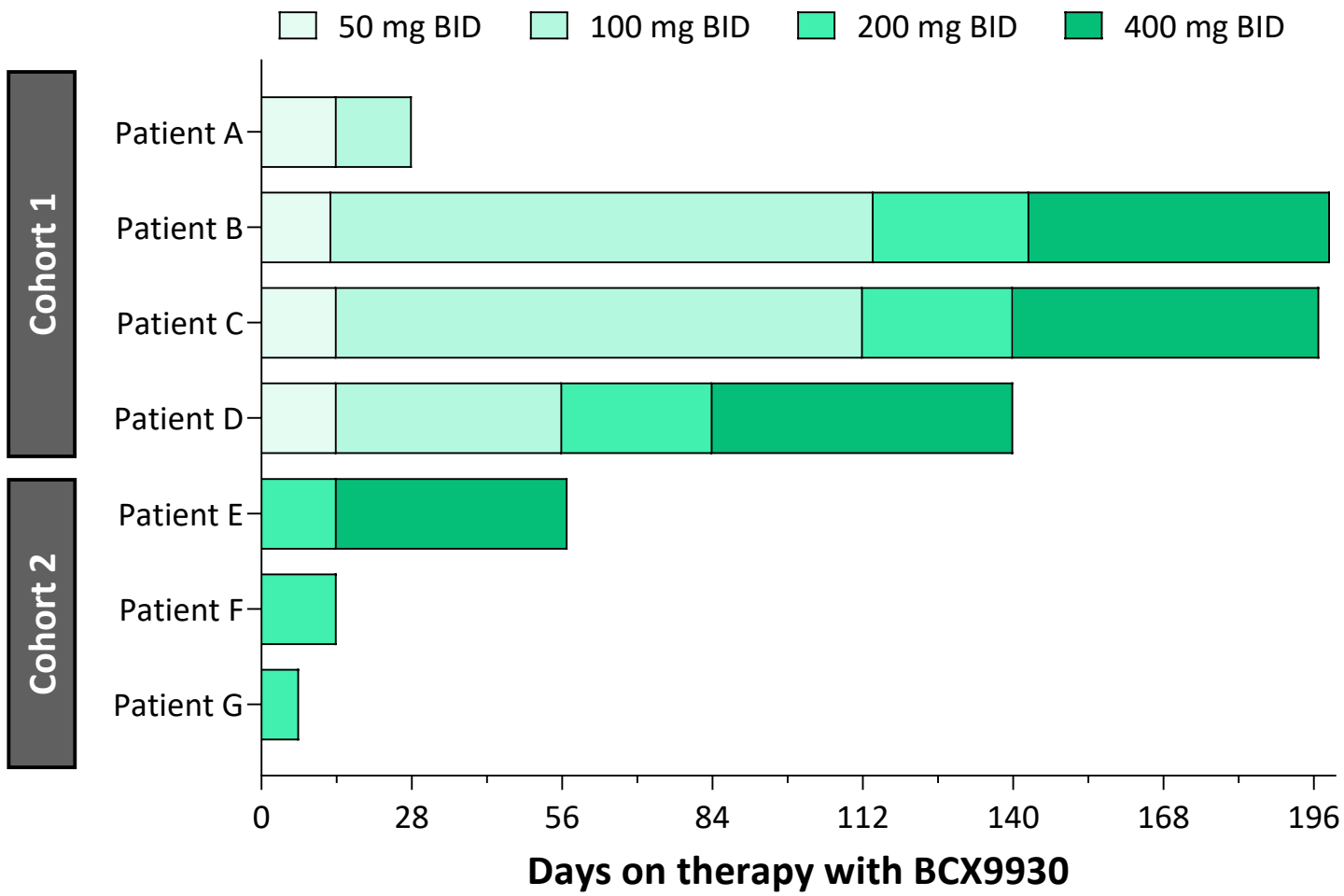
- Hb < 10 g/dL or blood transfusion within the last 12 months
- LDH ≥ 2 x ULN
- PNH clone size > 10%
- Platelet count > 30,000/µL
- Reticulocyte count > 100,000/µL

Treatment-naïve PNH Patients Had Severe Disease Prior to Treatment

Pre-treatment Characteristics	Cohort 1				Cohort 2		
Sequential Patient # in Cohort	*1*	*2*	*3*	*4*	*1*	*2*	*3*
Patient Code	*A*	*B*	*C*	*D*	*E*	*F*	*G*
PNH duration, years	8	4	4	5	2	5	1
Compromised bone marrow function	no	no	yes	no	yes	yes	yes
History of thrombosis, pulmonary HT or PNH renal injury	yes	yes	no	no	no	no	no
Lactate dehydrogenase (LDH), × ULN	9.8	11.0	3.7	6.9	4.2	4.6	3.8
Hemoglobin, g/dL	8.2	7.0	6.0	10.7	6.7	7.6	11.0
Units of RBC transfused in 52 weeks prior to screening	0	13	0	2	12	1	2
Reticulocytes, 10^3cells/µL	220	285	130	203	128	115	181
PNH erythrocyte (RBC) clone size, %	89	41	49	49	33	76	48
PNH RBC relative to PNH WBC, %	89	42	53	60	36	78	61

Laboratory values for LDH, reticulocyte count, total bilirubin and PNH erythrocyte clone size are average of available screening and baseline results. HT: hypertension.
Hemoglobin is last available value prior to Day 1 of BCX9930 administration. Study is ongoing – preliminary data.
Patients highlighted in green shading have progressed through at least 6 weeks of treatment on study at 400 mg BID
Patients with compromised bone marrow function have history of aplastic anemia or intermediate PNH

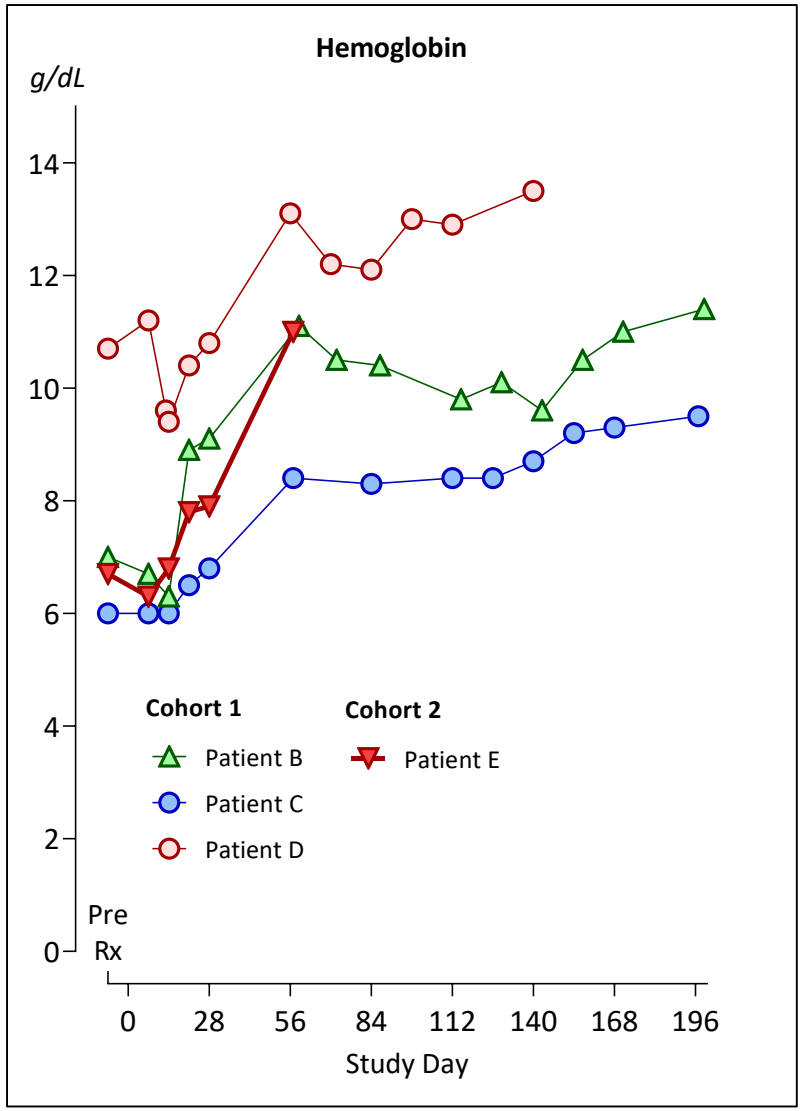
Duration of BCX9930 Treatment in PNH Patients



Data update from 4 patients on 400 mg BID an average of 53 days to date

Study is ongoing – preliminary data. Patients B – G remain on treatment in study
As disclosed in May 2020, Patient A discontinued due to an unrelated SAE

Meaningful Changes in Key Biomarkers Indicating Control of Hemolysis

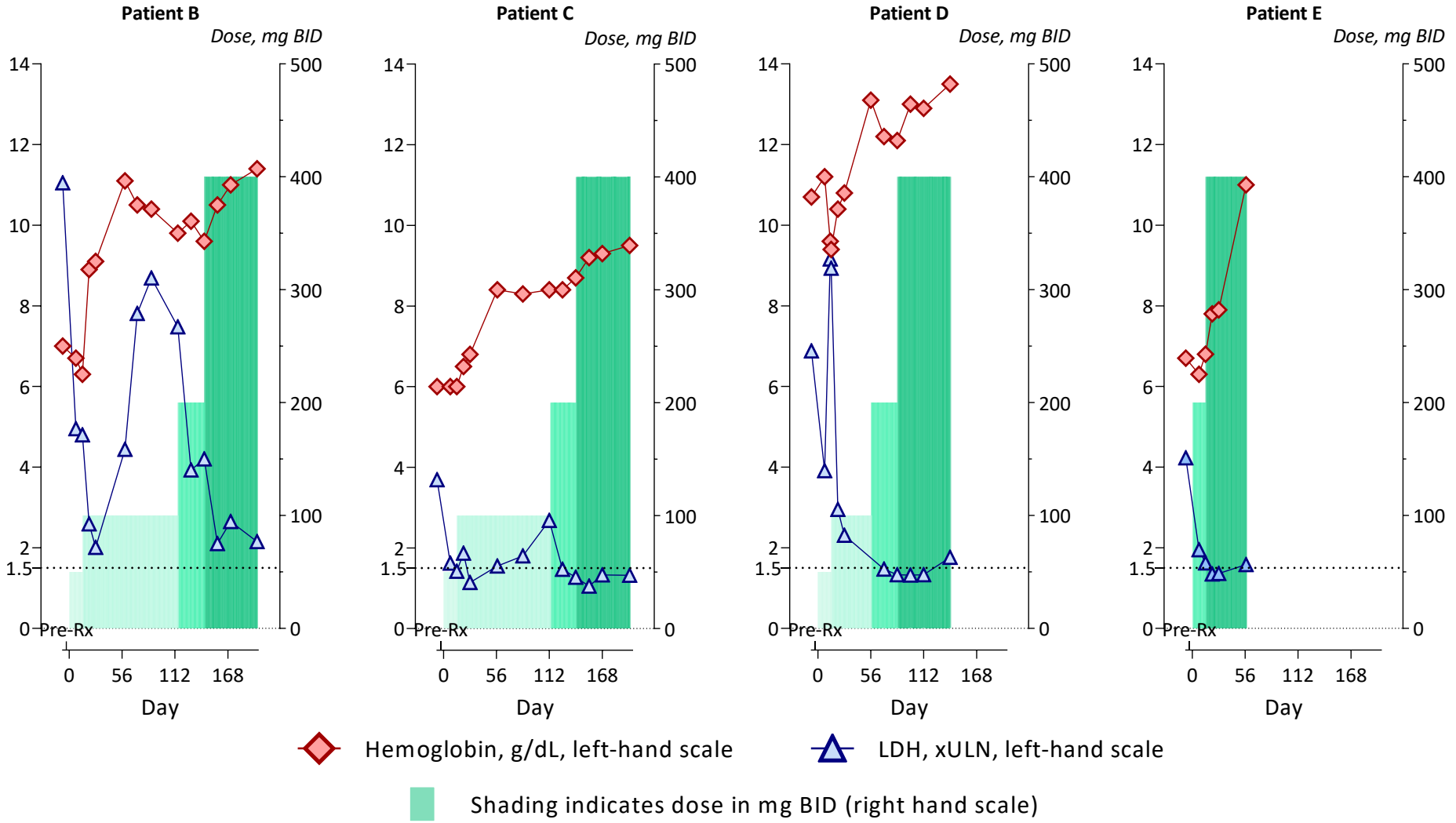


Patient	Duration at 400 mg BID	Hemoglobin g/dL		RBC Clone Size % of Granulocyte Clone Size		# of Transfusions @ 200/400 mg
		Pre-Rx	Most Recent	Pre-Rx	Most Recent	
B	56 days	7.0	11.4	42%	100%	0
C	57 days	6.0	9.5	53%	97%	0
D	56 days	10.7	13.5	60%	87%	0
E	43 days	6.7	11.0	36%	92%	0
Mean	***53 days***	***7.6***	***11.4***	***48%***	***94%***	***0***

- Mean increase in Hb from baseline of 3.8 g/dL
- Hb maintained at 400 mg BID without RBC transfusions
- Mean RBC PNH clone size relative to granulocyte clone size increased to 94% from 48% pre-Rx

Study is ongoing – preliminary data. One 2-unit RBC transfusion in Patient B on study day 15 after 50 mg BID x 14 d (previously reported).

BCX9930 Dose-response in Hemoglobin and LDH in PNH Patients



Study is ongoing – preliminary data

Hemolysis Biomarkers and Clinical Assessment Support Clinical Benefit of BCX9930 as Monotherapy in PNH

Clinical Data at 400 mg BID

Dose-dependent and clinically meaningful changes in key disease biomarkers were observed

- Mean hemoglobin increase from baseline was 3.8 g/dL
- Hb maintained at 400 mg BID without RBC transfusions (4/4)
- Mean RBC PNH clone size relative to granulocyte clone size increased from 48% pre-treatment to 94%, representing near-complete control of hemolysis
- Average LDH < 1.5 x ULN (3 of 4 patients)

Investigator-assessed clinical benefits

- All subjects treated assessed as benefiting from BCX9930 and continued on therapy

Study is ongoing – preliminary data

BCX9930 has been Safe and Well Tolerated in PNH Patients

Overall Safety

- No discontinuations due to related AEs
- No BCX9930-related serious AEs or safety signals
  - No safety signals in routine monitoring of vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry

Adverse Events

- The most common drug-related TEAE was mild-moderate headache lasting 1-3 days
- One patient had mild rash that resolved during continued BCX9930 dosing at 100 mg BID
- One patient had mild rash that is resolving during uninterrupted dosing after dose escalation to 400 mg BID
- One unrelated serious AE*

*Study is ongoing – preliminary data. *Unrelated SAE previously reported, primary disseminated VZV infection in a non-immune subject taking corticosteroids, fatal.*

Next Steps for BCX9930 Development

Q32020

Data update from BCX9930 200/400 mg treatment-naïve PNH dose-ranging study

Q42020

Complete dose-ranging in C5 inhibitor naïve patients with 500 mg BID

Meet with regulators to discuss advanced development program

Data update from BCX9930 200/400 mg inadequate responder PNH dose-ranging study by year-end

2021

Begin multiple advanced development **clinical trials** in hematology and nephritis diseases of the alternative pathway

Goal = Oral Monotherapy in Multiple Complement-mediated Diseases