Second Quarter 2020 Results Call
Corporate Update & Financial Results

August 6, 2020

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Agenda

- Corporate Update:

  Jon Stonehouse – President, Chief Executive Officer

- Global Berotralstat Launch Update:

  Charlie Gayer – Chief Commercial Officer

  Megan Sniecinski – Chief Business Officer

- BCX9930 and Galidesivir Clinical Update

  Dr. Bill Sheridan – Chief Medical Officer

- Financial Update

  Anthony Doyle – Chief Financial Officer

- Summary and Q&A

Global Berotralstat (BCX7353) Launch Update:

Charlie Gayer – Chief Commercial Officer

Megan Sniecinski – Chief Business Officer

Coming Soon: Orladeyo™

orladeyo™
(berotralstat) 150 mg capsule

Clinical Update:

Dr. Bill Sheridan – Chief Medical Officer

Factor D: Outstanding Target for Complement-mediated Diseases

Factor D is an ideal target:	Application to BCX9930 Development:
Required for the alternative pathway (AP) to work	Doses of BCX9930 that block Factor D will inhibit the AP independent of the disease setting
Target is the same in PNH, nephritis, and other AP diseases	Proof of concept in PNH provides POC for other diseases of the alternative pathway
Circulating Factor D levels are the lowest of any complement pathway enzyme	Less drug required for inhibition compared to other complement targets
Levels do not increase with inflammatory illnesses	No dose adjustment when patients get illnesses like influenza
Unique enzyme structure enables design of inhibitors with better specificity against other serine proteases	Can lead to a better safety margin

Targeting Overactive Alternative Pathway Could Treat Many Complement-mediated Diseases

Pathology of Renal Diseases Associated with Dysfunction of the Alternative Pathway of Complement: C3 Glomerulopathy and Atypical Hemolytic Uremic Syndrome (aHUS)

Sanjeev Sethi, MD, PhD[1] Fernando C. Fervenza, MD, PhD[2]

Causes of Alternative Pathway Dysregulation in Dense Deposit Disease

Yuzhou Zhang, Nicole C. Meyer,* Kai Wang,† Carla Nishimura,* Kathy Frees,* Michael Jones,* Louis M. Katz,‡ Sanjeev Sethi,§ and Richard J.H. Smith*‖*

REVIEW
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Anti-complement Treatment for Paroxysmal Nocturnal Hemoglobinuria: Time for Proximal Complement Inhibition? A Position Paper From the SAAWP of the EBMT

Antonio M. Risitano[1,2], Serena Marotta[1,2], Patrizia Ricci[1], Luana Marano[1], Camilla Frieri[1], Fabiana Cacace[1], Michela Sica[3], Austin Kulasekararaj[3,4], Rodrigo T. Calado[5], Phillip Scheinberg[6], Rosario Notaro[3†] and Regis Peffault de Latour[2,7†] on behalf of the Severe Aplastic Anemia Working Party of the European group for Bone Marrow Transplantation*

BCX9930 28-day PNH Proof of Concept Study Design

Key Outcome Measures

- **LDH, hemoglobin**
- **Safety**
- **PK**
- **PD**

	Total of 28 days of BCX9930 dosing	
	Period 1 days 1-14	Period 2 days 15-28
Subjects with PNH who are naïve to C5-INH treatments: BCX9930 monotherapy		
Cohort 1: n = up to 4*	50 mg BID days 1-14	100 mg BID days 15-28
Cohort 2: n = up to 4	200 mg BID days 1-14	400 mg BID days 15-28
Subjects with PNH with poor response to C5-INH: BCX9930 plus continued C5-INH		
Cohort 2: n = up to 4	200 mg BID days 1-14	400 mg BID days 15-28

Subjects benefiting from study drug may continue on treatment

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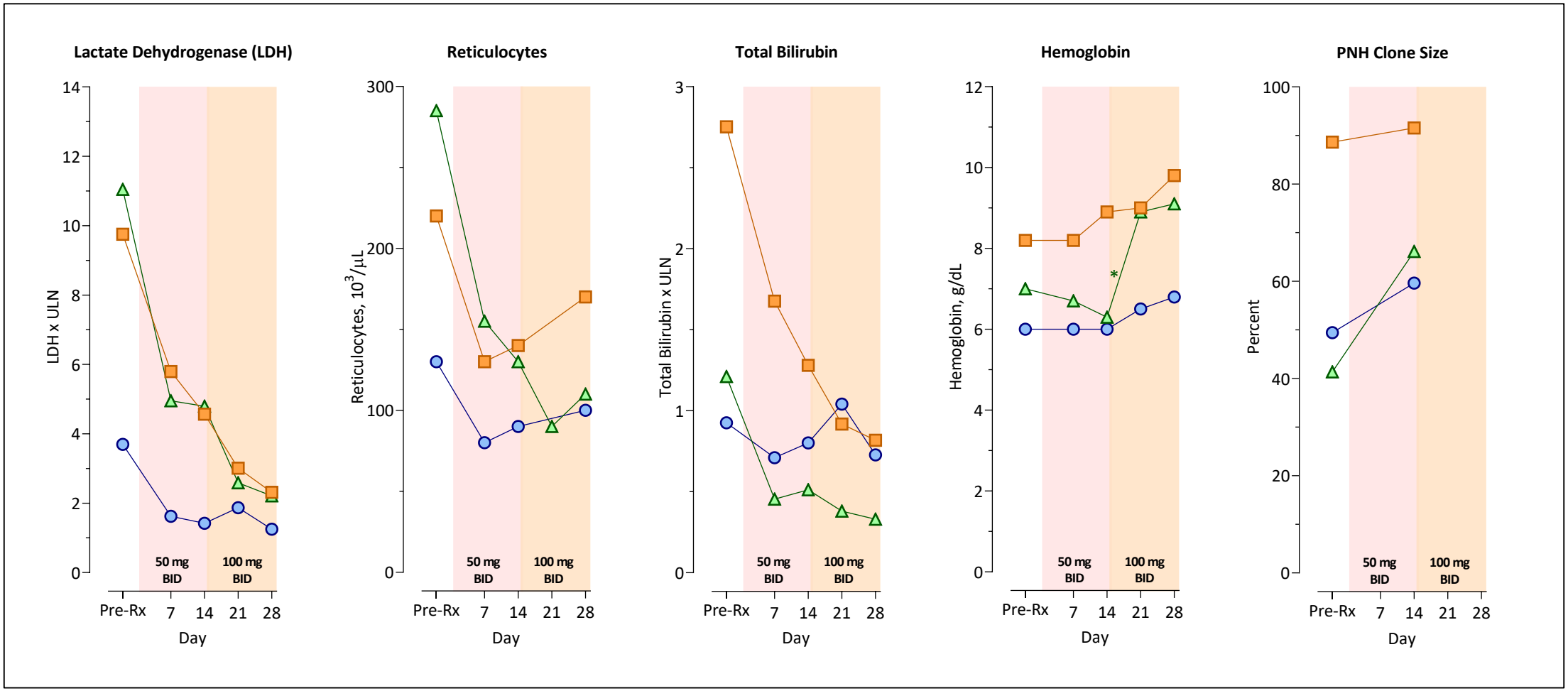
** Cohort 1 enrolment for subjects naïve to C5-INH is completed. Per protocol, these Cohort 1 subjects have been up-titrated to higher doses in the extension period.*

Treatment-naïve PNH Patients had Severe Disease

Pre-treatment Characteristics	Subject 1	Subject 2	Subject 3
PNH duration, years	8	4	3
History of aplastic anemia	no	no	yes
History of thrombosis	yes	no	no
LDH, IU/L	2205	2497	835
LDH × ULN	*9.8*	*11.0*	*3.7*
Hemoglobin, g/dL	8.2	7.0	6.0
Reticulocytes, 10^3cells/µL	220	285	130
Total bilirubin, mg/dL	3.33	1.47	1.12
PNH type III erythrocyte clone size, %	89	41	49
Units of RBC transfused in 52 weeks prior to screening	0	13	0
Units of RBC transfused in 12 weeks prior to screening	0	2	0

Laboratory values for LDH, reticulocyte count, total bilirubin and PNH type III erythrocyte clone size are average of available screening and baseline results.
Hemoglobin is last available value prior to Day 1 of BCX9930 administration. Study is ongoing – preliminary data.

Dose-dependent Improvement Across Key Indicators in Treatment-naïve PNH Subjects Receiving BCX9930 Monotherapy



Study is ongoing – preliminary data. Assays pending for RBC clone size on day 28.
Asterisk indicates RBC transfusion in Subject 2 on day 15

BCX9930 Data Provides Strong Support for Oral Monotherapy in PNH

Safety & Tolerability in PNH, n=3

- BCX9930 has been safe and generally well-tolerated in cohort 1 at low doses of 50 mg bid days 1-14 followed by 100 mg bid days 15-28
- No BCX9930-related serious adverse events
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry
- One death unrelated to study drug following 28 day study period
- 3/3 subjects had moderate headache resolving in <1-3 days soon after starting study drug
- No rash observed

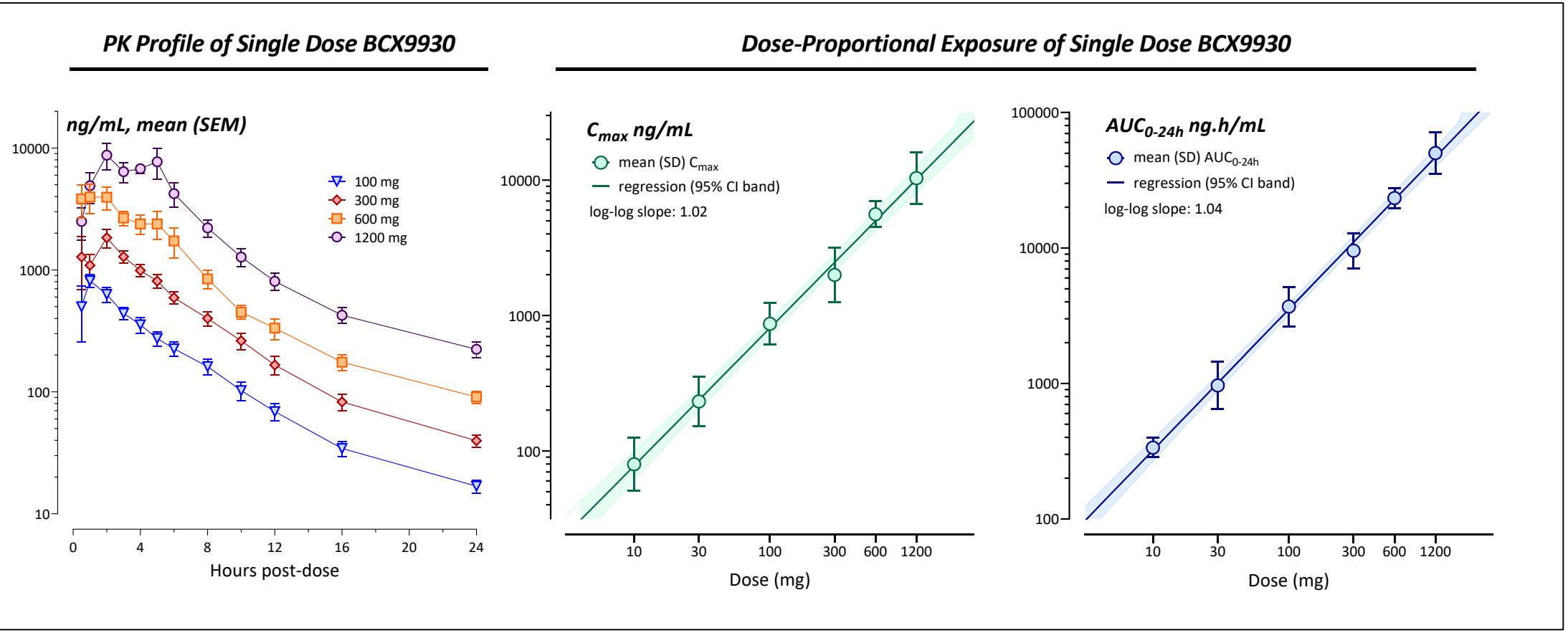
Activity in PNH at low doses, n=3

- Prompt and sustained reductions in LDH (3/3) and reticulocytes (2/3)
- Increasing PNH clone size and Hb
- Investigator assessed clinical benefit in 3/3 patients, all continued to long-term extension

Next steps

- Opened 200/400 mg bid cohort for C5-inhibitor naïve patients after completing cohort 1, data expected Q3 2020
- Data from C5-inhibitor poor responders in 200/400 mg bid cohort expected by YE 2020

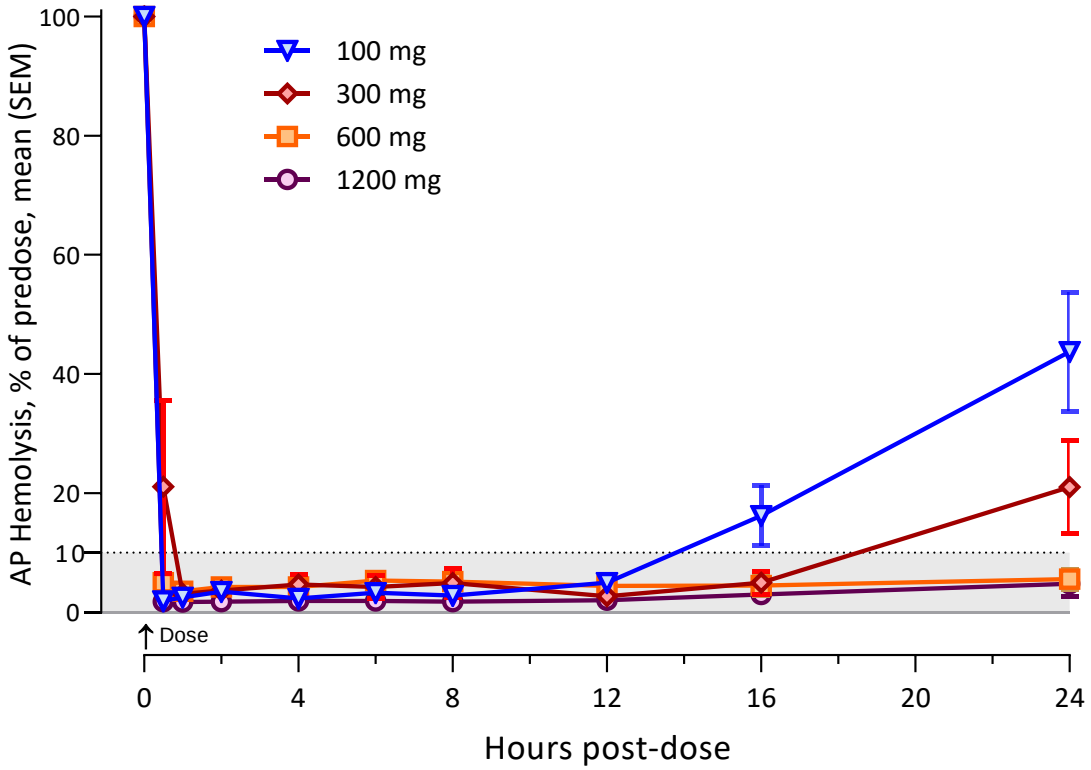
Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



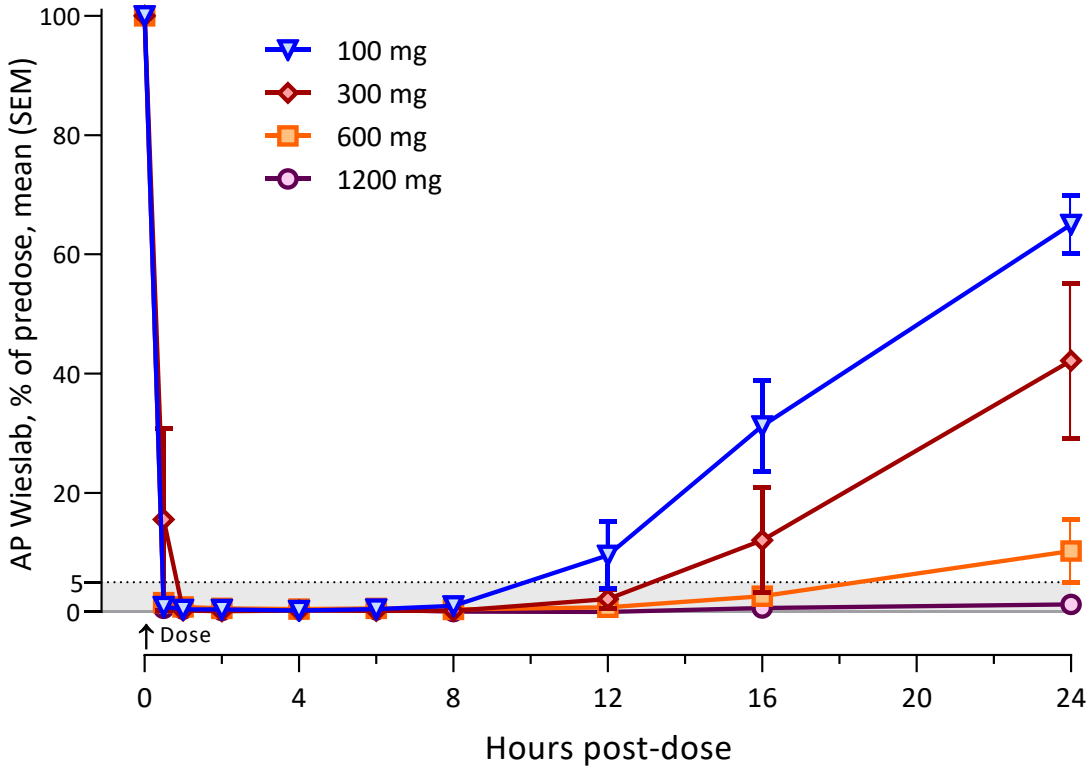
Suppression of AP Activity After Single Oral Doses of BCX9930

Alternative pathway complement activity in healthy subjects : oral BCX9930 single dose

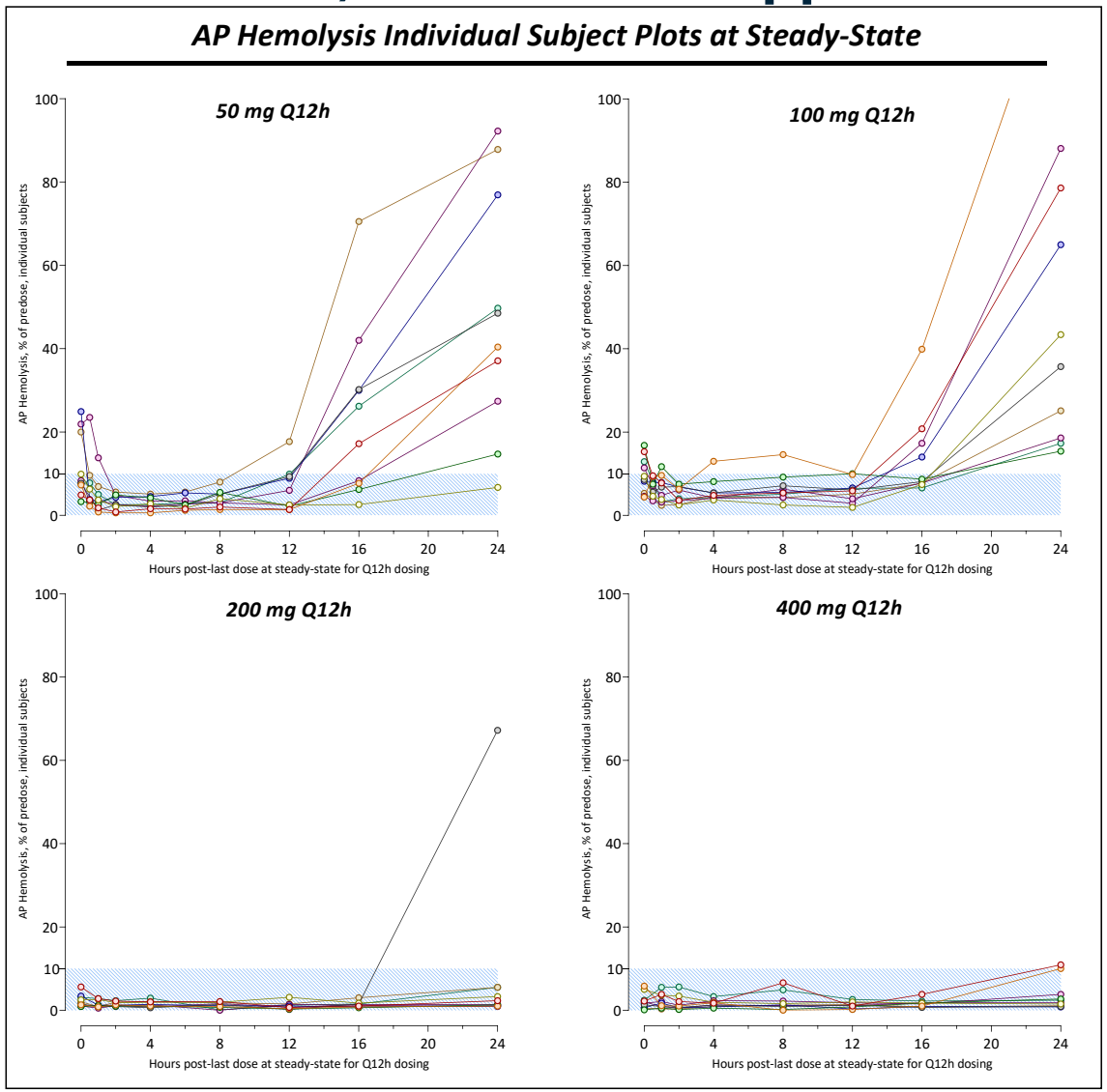
Assay: AP Hemolysis

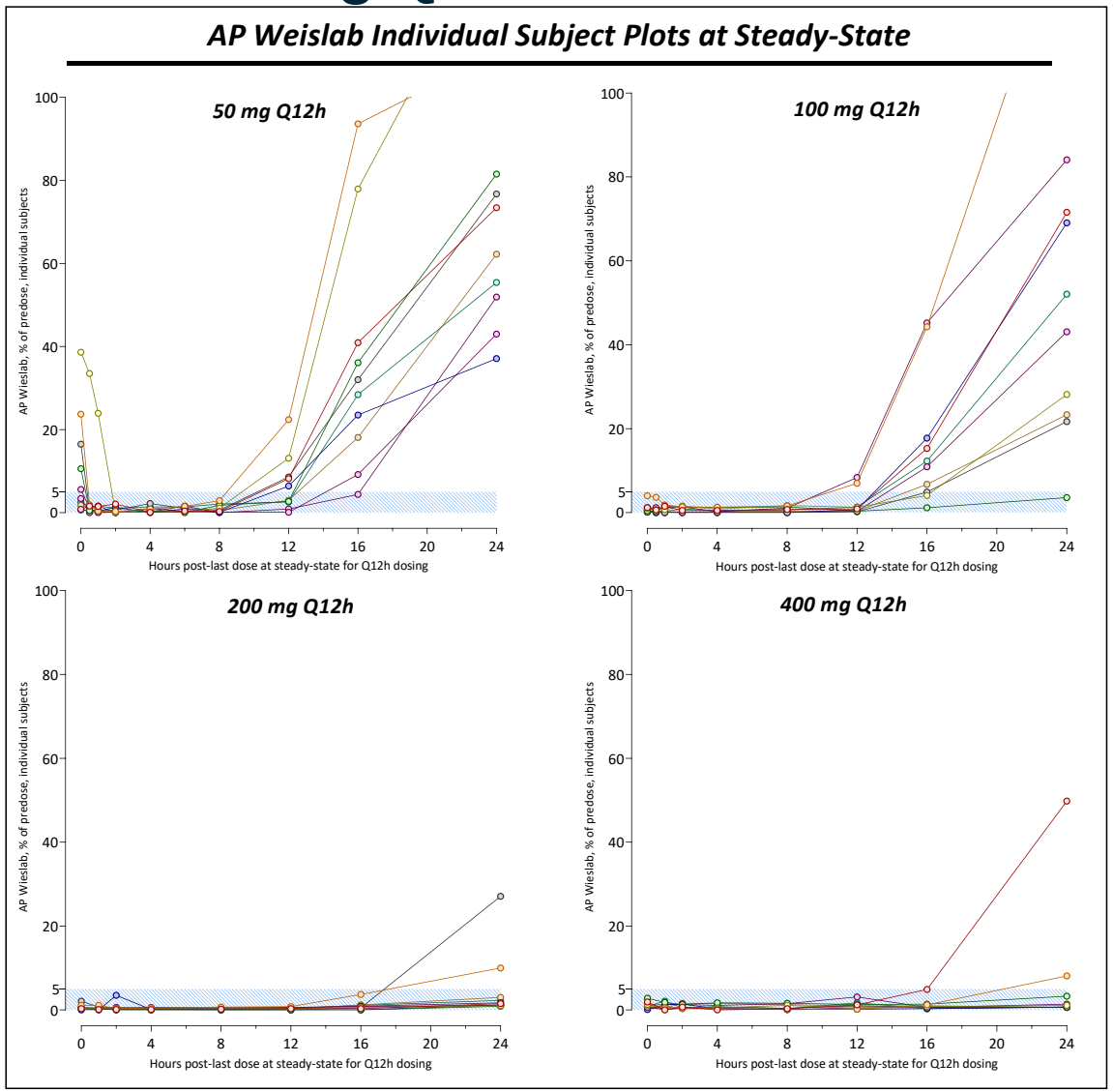


Assay: AP Wieslab



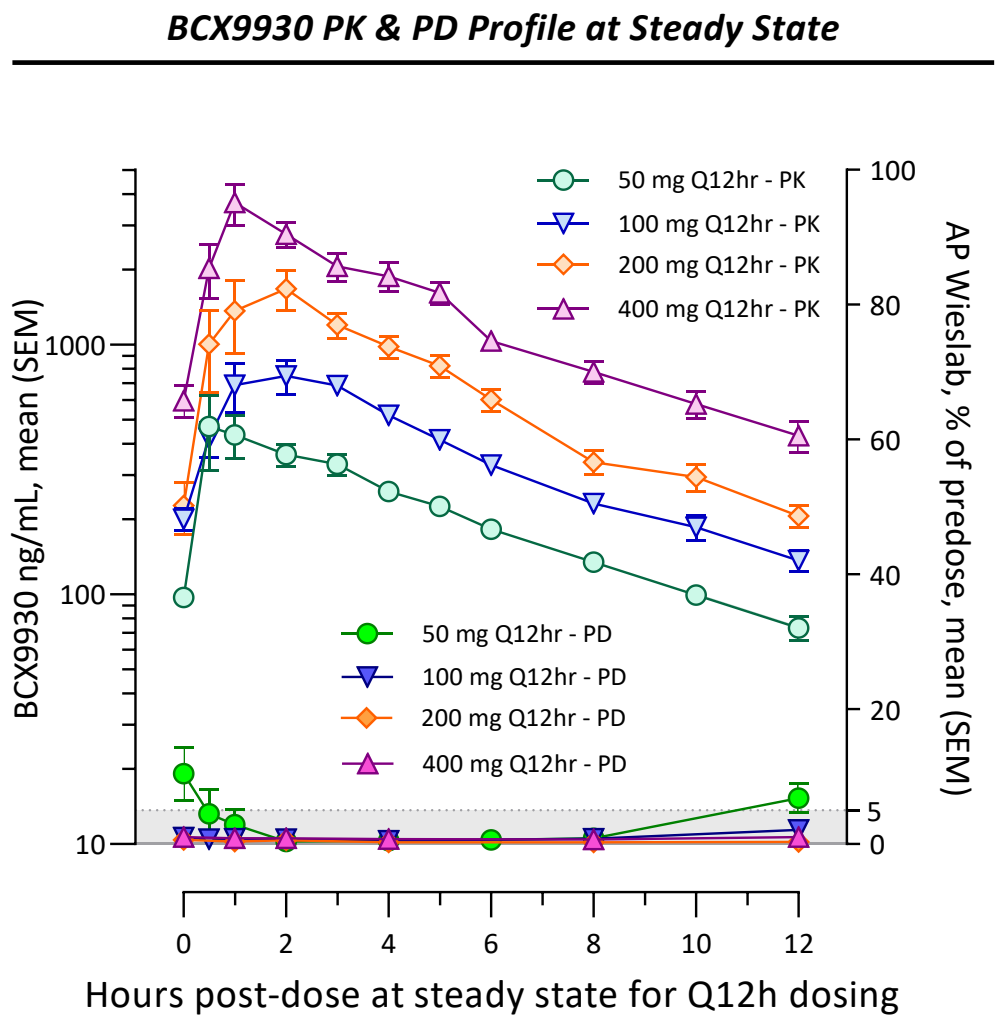
Clear Dose-response in AP Inhibition – Consistent, Sustained Suppression at 200/400 mg Q12h

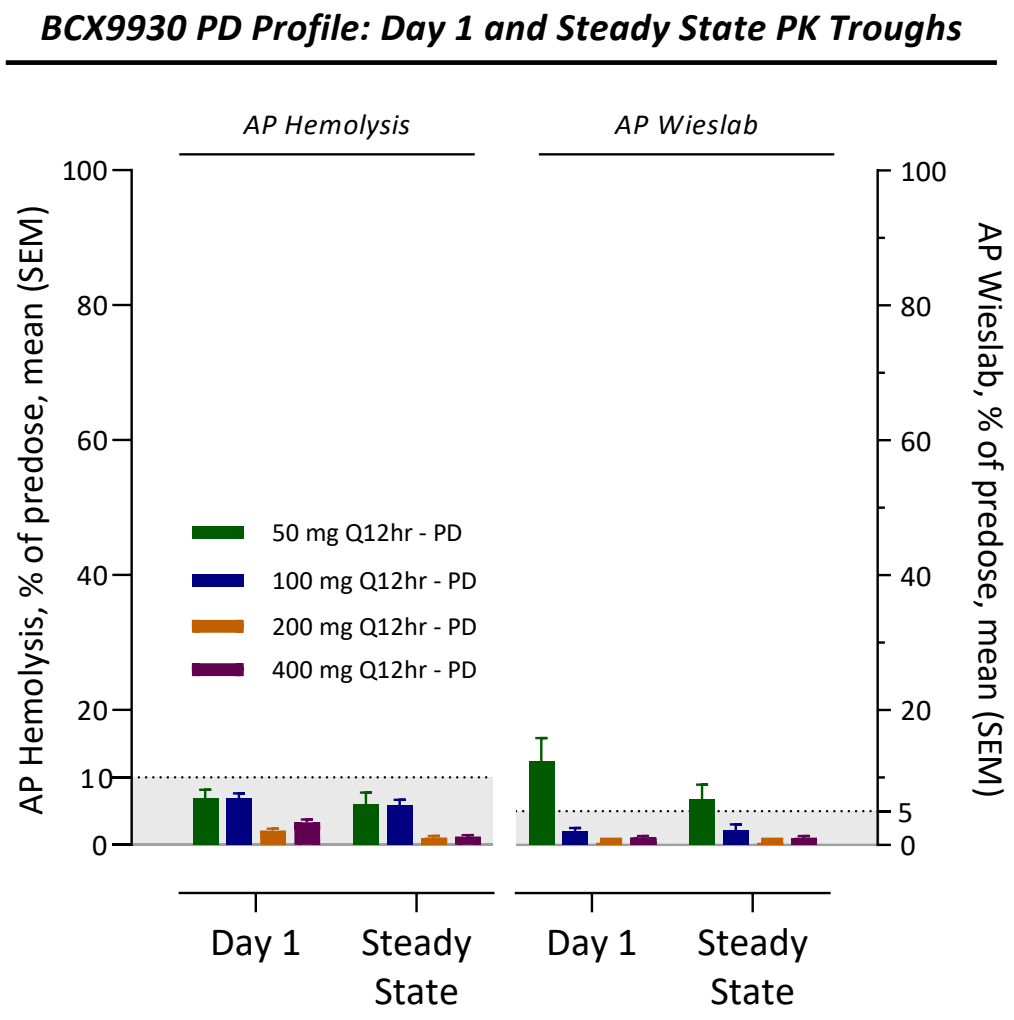




Last dose of BCX9930 on Q12 hr schedule was administered at time 0, and results through 24 hours post-dose are shown

Greater Exposure at 200/400 mg with >98% Sustained Alternative Pathway Suppression in Both Assays





Successful BCX9930 SAD/MAD Supports Monotherapy for Diseases of the Alternative Pathway

Safety & Tolerability: Healthy Subjects

- Study drug was safe and generally well-tolerated at all doses
- No serious adverse events
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry
- Benign rash in majority of MAD subjects was self-limited and resolved within a median of 5 days of onset
- No dose-related safety signals observed

PK/PD in Healthy Subjects

- Linear, dose-proportional exposure
- Dose-related suppression of AP of complement functional activity
- > 98% inhibition of AP in both AP Wieslab and AP hemolysis assays at steady-state dosing for doses of 200 mg Q12h and 400 mg Q12h

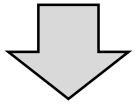
Next Steps

- Test supratherapeutic doses to finish SAD/MAD
- Explore once-daily dosing

Galidesivir Clinical Trial in COVID-19 Enrolling at 4 Sites in Brazil

Part 1 (n=24)

Cohort 1 *GVR n=6 , PBO n=2*	10 mg/kg then 2 mg/kg q12h×13
Cohort 2 *GVR n=6 , PBO n=2*	10 mg/kg then 5 mg/kg q12h×13
Cohort 3 *GVR n=6 , PBO n=2*	20 mg/kg then 5 mg/kg q12h×13



Part 2 - Randomized 2:1 (GVR:PBO)

Cohort	Dose selected from Part 1 (n=42)

Key Outcome Measures

- Safety
- PK
- Viral Load Reduction
- Changes in clinical signs and symptoms

Financial Update:

Anthony Doyle– Chief Financial Officer

Cash position & 2020 guidance (in millions)

Cash & investments at December 31, 2019	$138
Cash & investments at March 31, 2020	$115
Cash & investments at June 30, 2020	$192
Senior credit facility	$50
REVISED FY 2020 GUIDANCE	
Net operating cash utilization	$150-165
Operating expenses [A]	$180-195

A - Excludes equity-based compensation.

Thank You…
Questions and Answers