H.C. Wainwright Global Investor Conference

Jon Stonehouse
Chief Executive Officer

Dr. Bill Sheridan
Chief Medical Officer

September 14, 2020

Forward-Looking Statements

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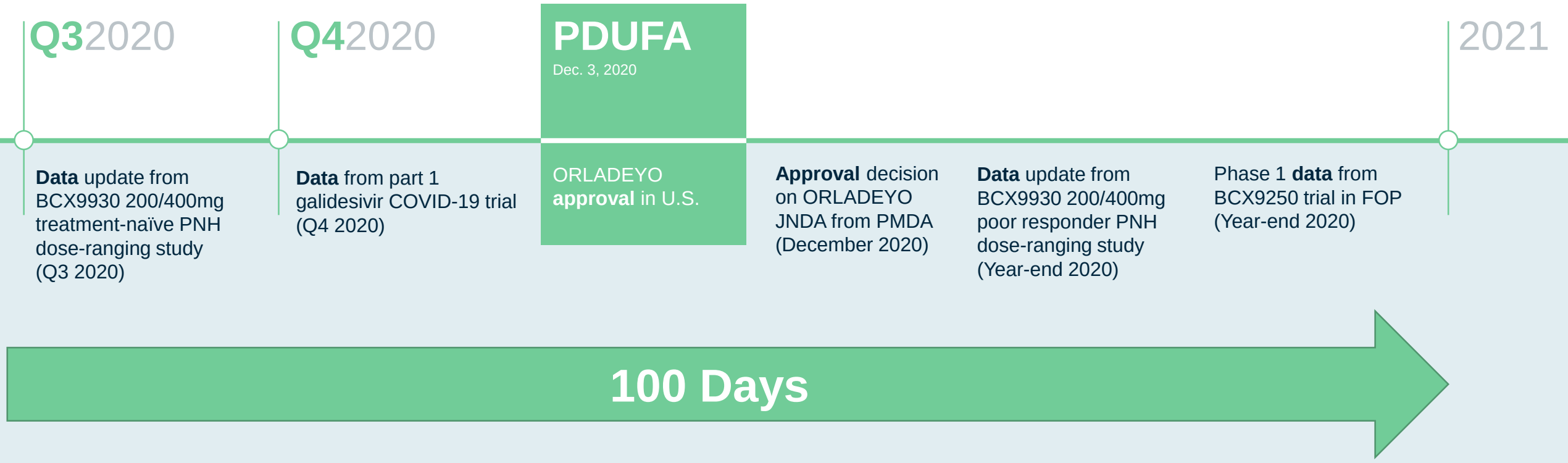
Six Major Program Updates Expected in Next 100 Days



Coming Soon: Orladeyo™

orladeyo™
(berotralstat) 150 mg capsule

Ready for Successful Commercial Launch

- Sales force hired + trained
  - Average 20 years of experience, including 8 in rare disease
  - Top performers
- Medical Affairs team deployed and engaging with the KOL community
- Patient access/support systems in place
- Robust dual-source supply chain to support commercial launch

COVID-19 Antiviral Status Update: Galidesivir (BCX4430)

New $47M of NIAID Contract Awards to Support:

- Completion of Parts 1 & 2 of ongoing clinical trial of galidesivir in Brazil
- Conducting a phase 2 trial of galidesivir in non-hospitalized COVID-19 patients at high risk for developing severe disease and complications
- Conducting a clinical pharmacology trial of galidesivir to determine appropriate dosing in patients with renal impairment
- Increasing the supply of galidesivir

- Part 1 of trial in Brazil has advanced to final cohort
- Data from Part 1 + decision on dose for Part 2 expected in Q4 2020

- Galidesivir has shown activity against >20 RNA viruses in 9 different families, including coronaviruses[1]
- Animal and *in vitro* studies against SARS-CoV-2 ongoing

$ 129 M of program support to date:

National Institute of Allergy and Infectious Diseases

BIOMEDICAL ADVANCED RESEARCH AND DEVELOPMENT AUTHORITY
BARDA

[1] Warren, T. K. *et al. Protection against filovirus diseases by a novel broad-spectrum nucleoside analogue BCX4430. Nature* ***508, 402-405, doi:10.1038/nature13027 (2014)***

Goal for BCX9930: Oral Factor D Inhibitor as Monotherapy for Multiple 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

Significant Unmet Need in PNH Due to Uncontrolled Hemolysis

Anemia and PNH Symptoms Persist in Many Patients Despite C5 Inhibitor Treatment

C5 Inhibitor	Hb not Stabilized	Fatigue	Hemoglobinuria	Dyspnea
Ravulizumab	**32%**	29%	10%	14%
Eculizumab	**35%**	30%	9%	14%

J. W. Lee *et al.*, *Blood* **133**, 530-539 (2019)

Extravascular Hemolysis Develops in Most PNH Patients with C5 Inhibitor Treatment

- *All patients treated with eculizumab had opsonized RBCs* *C3+ RBC: 41/41 (100%)*
- *Most patients had persistent hemolysis*

A. M. Risitano *et al.*, *Blood* 113, 4094-4100 (2009)

- ***Effective Alternative Pathway complement inhibition with BCX9930 will control both intravascular and extravascular hemolysis regardless of bone marrow function****

*56% of >4,000 patients in the PNH registry had aplastic anemia with PNH, with impaired bone marrow function

H. Schrezenmeier *et al.*, *Ann Hematol* 99, 1505-1514 (2020)

Factor D Inhibition in PNH Offers the Advantage of Controlling Both Intravascular (IVH) and Extravascular (EVH) Hemolysis

Goal of Total Hemolytic Control

IVH Control

↓ LDH

+

EVH Control

↓ Opsonized RBCs

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)

Goal of Ongoing Dose-ranging Study: Choose Optimized Dose for Advanced Development as Monotherapy

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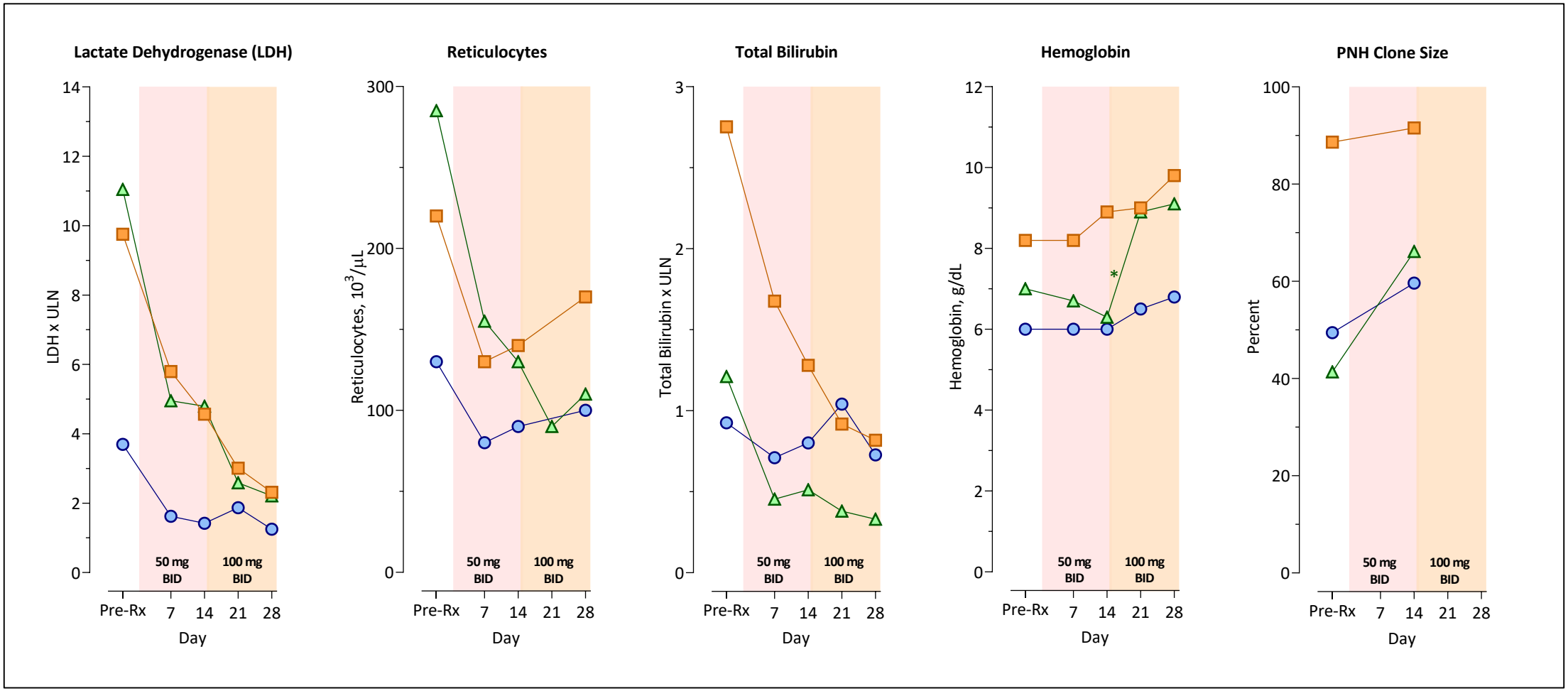
** Cohort 1 enrolment for subjects naïve to C5-INH is completed. Per protocol, these Cohort 1 subjects have been up-titrated to higher doses in the extension period.*

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

- 200/400 mg bid cohort for C5-inhibitor naïve patients, data update expected Q3 2020
- C5-inhibitor poor responders in 200/400 mg bid cohort, data update expected by YE 2020

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.

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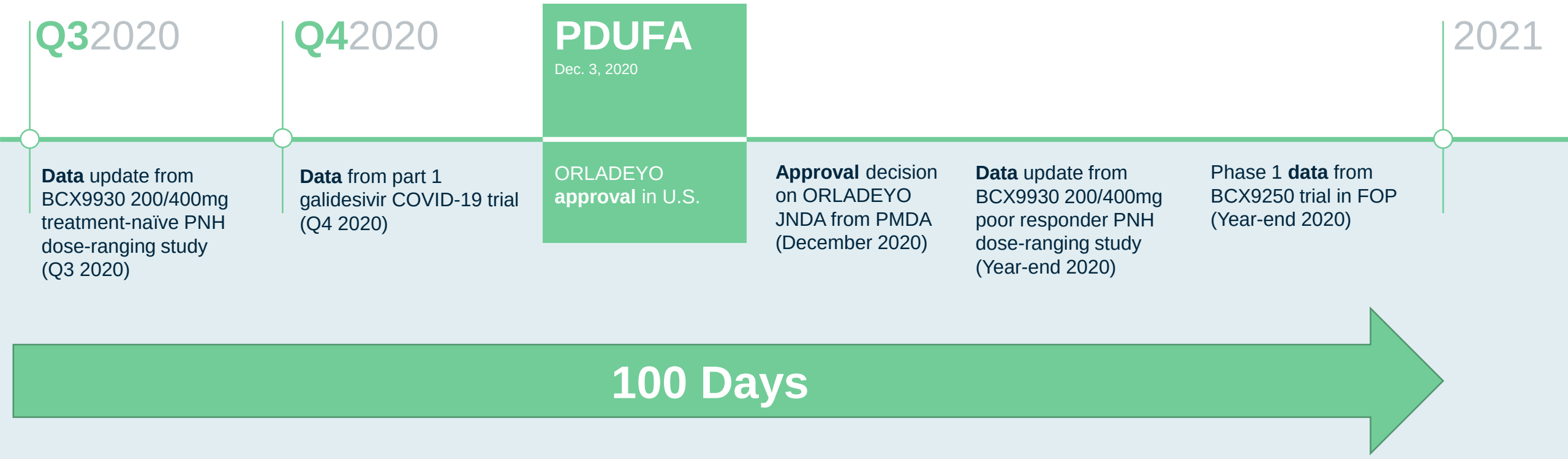

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