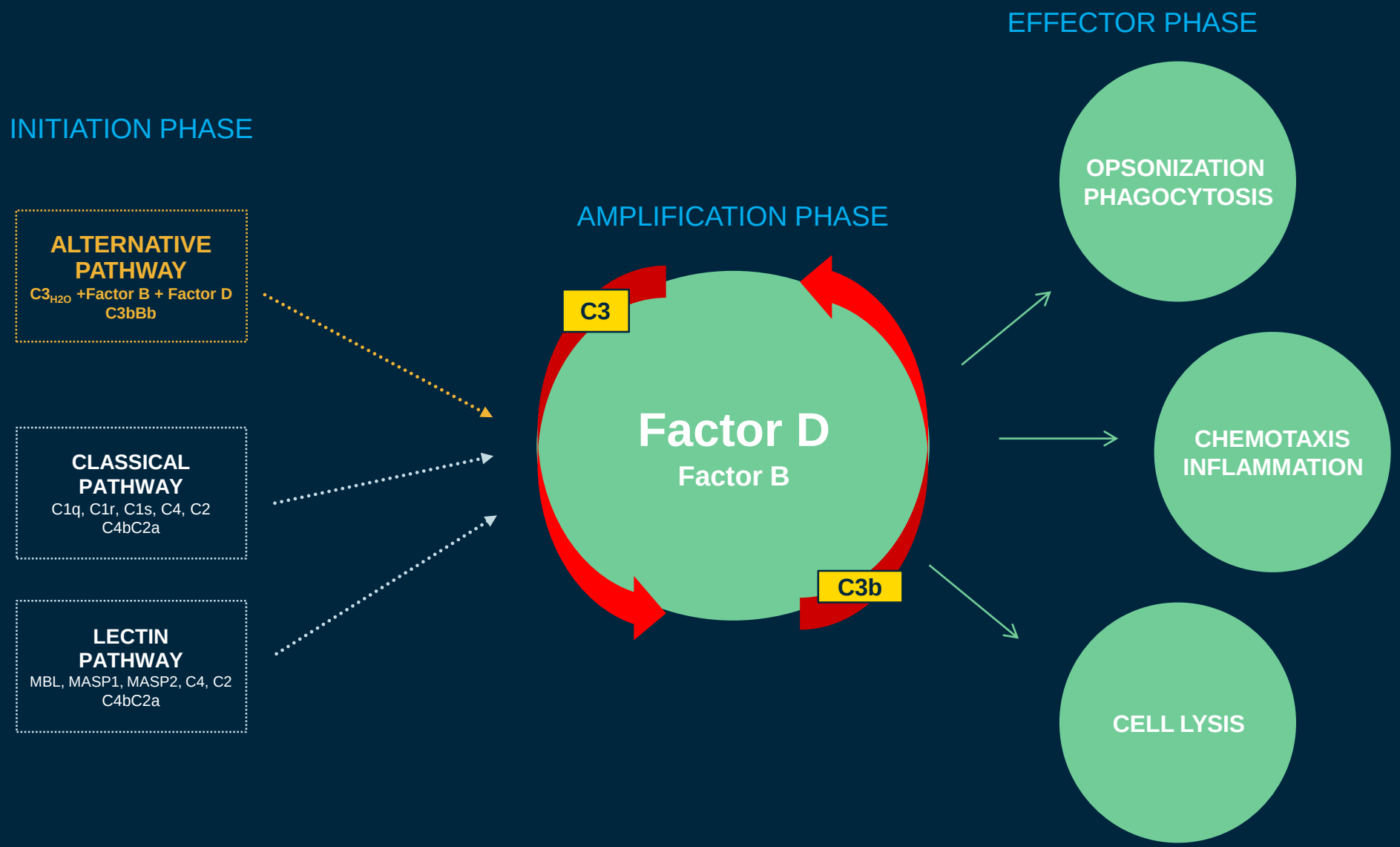
# BCX9930
# Phase 1 Update Call

October 28, 2019

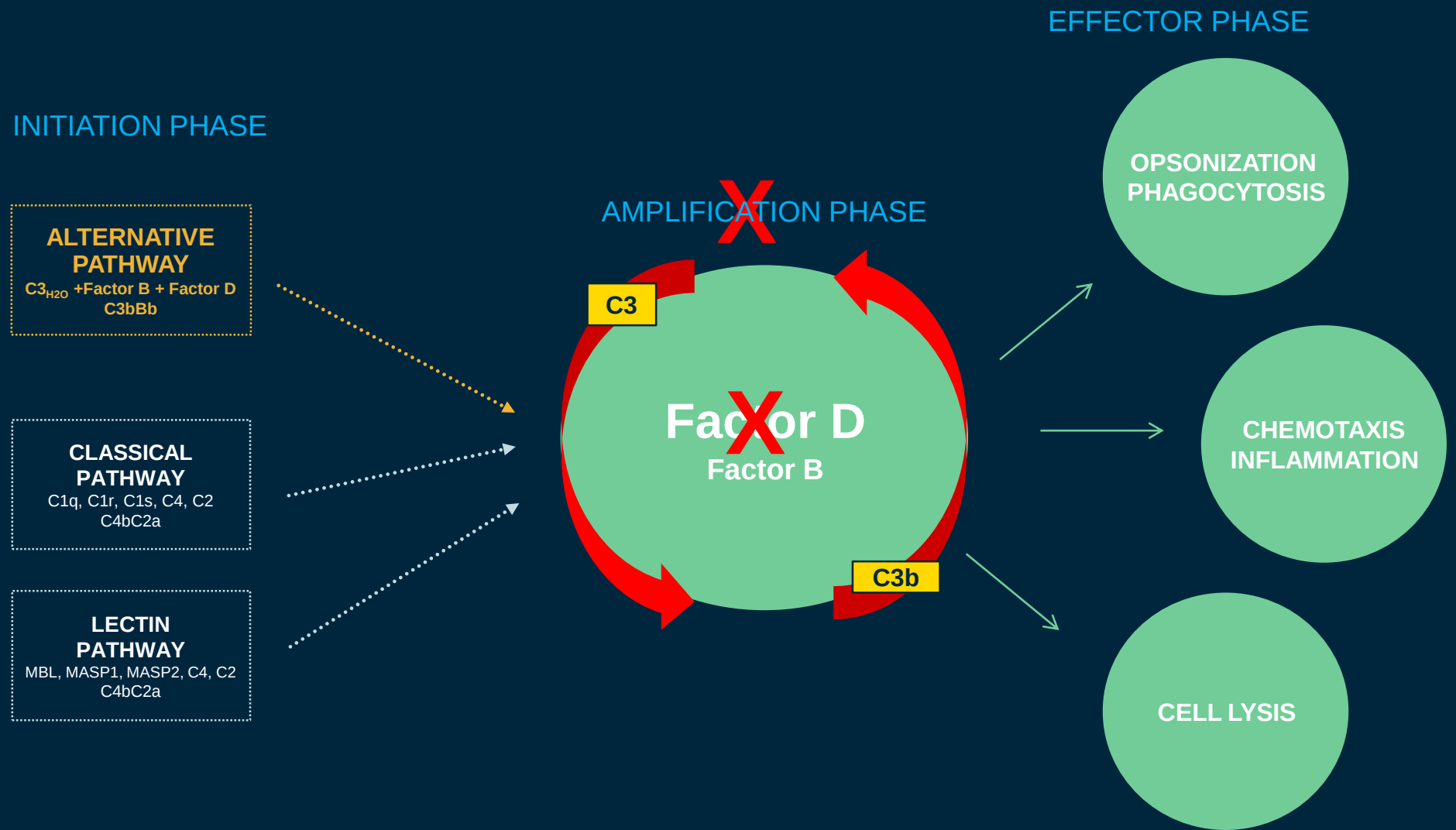
# Forward-Looking Statements

BioCryst's presentation may contain forward-looking statements, including statements regarding future results, unaudited and forward-looking financial information and company performance or achievements. These statements are subject to known and unknown risks and uncertainties which may cause our actual results, performance or achievements to be materially different from any future results or performances expressed or implied in this presentation. You should not place undue reliance on the forward-looking statements. For additional information, including important risk factors, please refer to BioCryst's documents filed with the SEC and located at http://investor.shareholder.com/biocryst/sec.cfm

# Factor D Plays a Key Role in Amplifying Complement Activation



# Targeting Factor D, the Rate Limiting Enzyme in the Alternative Pathway, Prevents Formation of Functional C3 Convertase Leading to Inhibition of Alternative Pathway Activity



# BCX9930 Phase 1 Trial Design & Progress

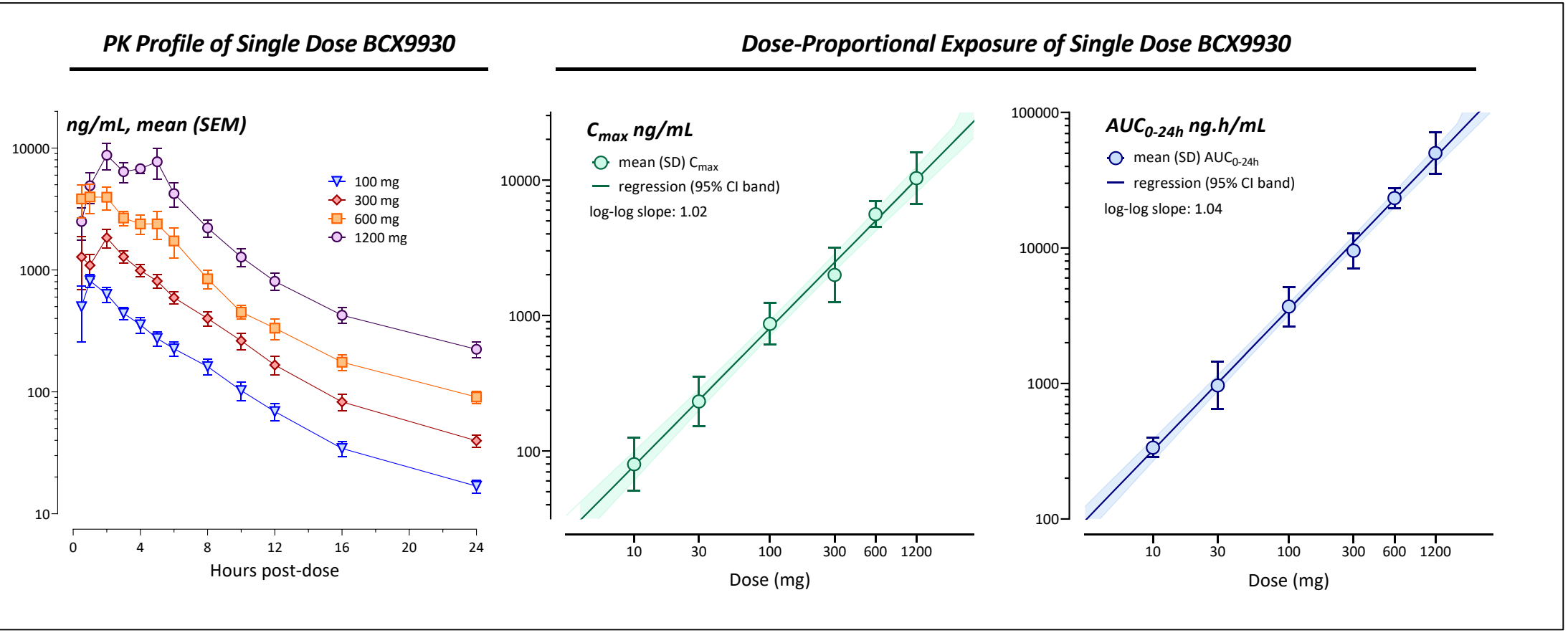
**Part 1 – Single ascending dose**
- Healthy subjects
- PK & PD
- Safety and tolerability
- 8 subjects per cohort
  6:2 active : placebo
- 6 dose levels
- Completed

**Part 2 – Multiple ascending dose**
- Healthy subjects
- PK & PD
- Safety and tolerability
- 12 subjects per cohort
  10:2 active : placebo
- Multiple dose levels
- Ongoing

**Part 3 – Proof of concept in PNH patients**
- Poor responders to eculizumab or ravulizumab, or naïve to treatment
- Up to 16 patients total
- Multiple dose levels

- Part 1 : SAD completed with cohorts from 10 to 1200 mg
- Part 2 : Two MAD cohorts completed with 50 and 100 mg Q12hr x 7 days
- Part 3 : PNH proof of concept data expected 1H 2020

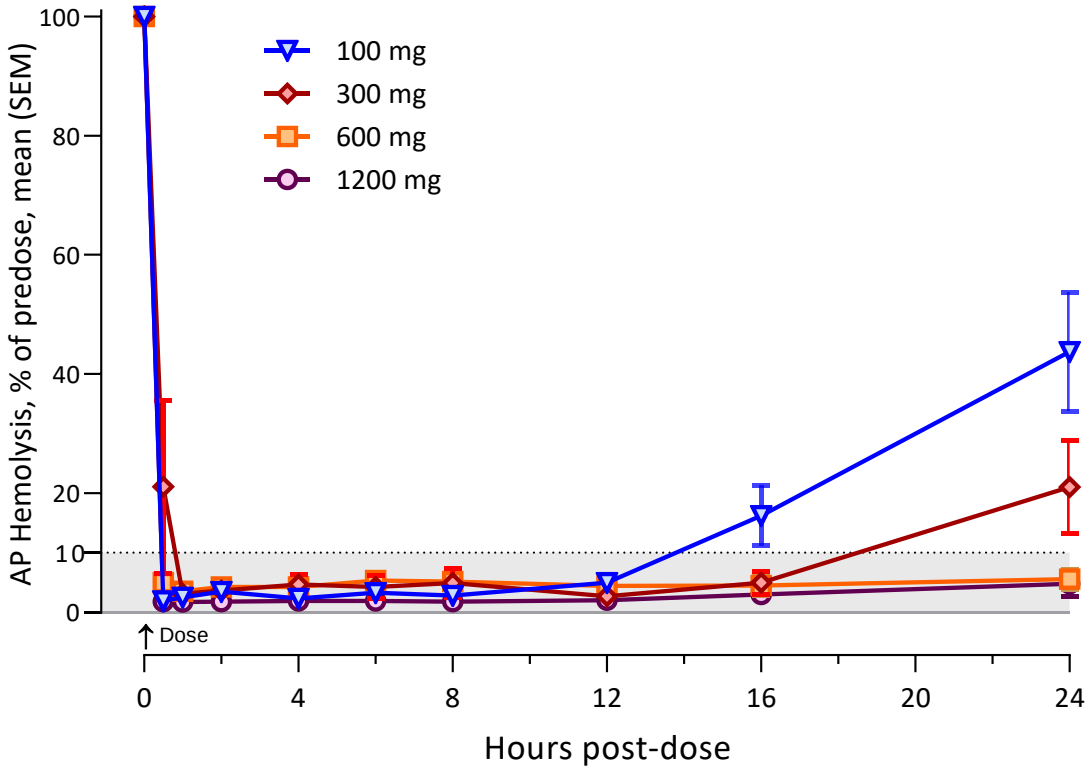
# Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



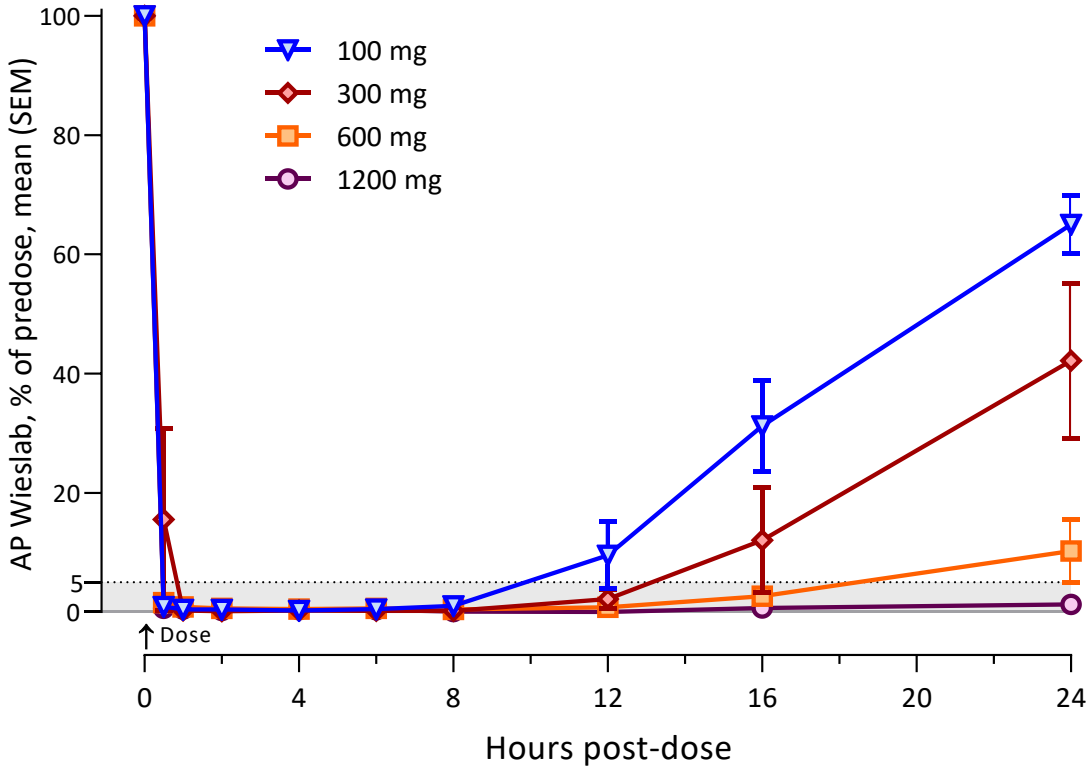
# Suppression of AP Activity after Single Oral Doses of BCX9930

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

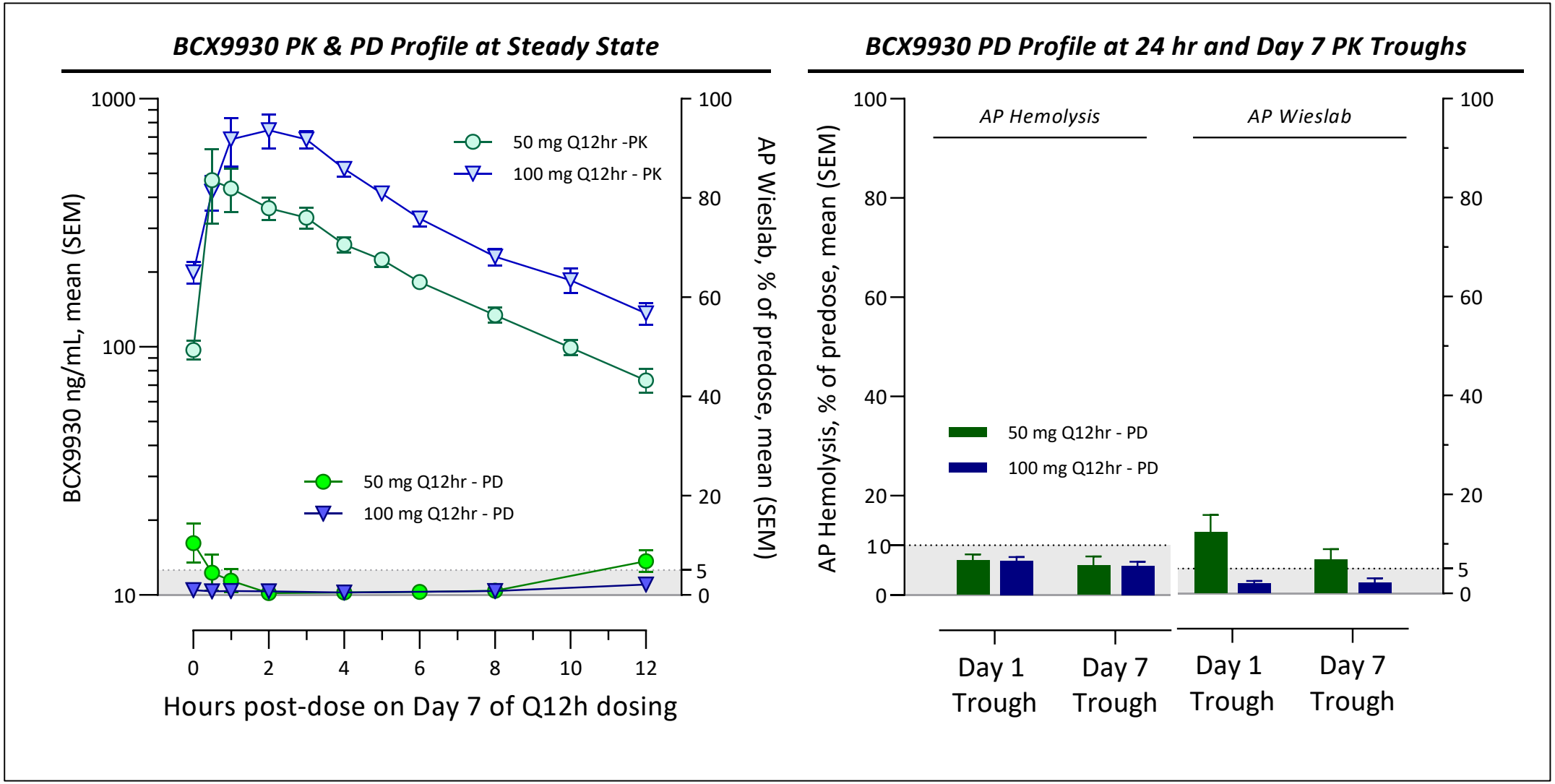
*Assay: AP Hemolysis*




*Assay: AP Wieslab*



# Steady State PK and PD with Q12hr Dosing of BCX9930



# BCX9930 Phase 1 Trial: Summary

## Safety & Tolerability

- Safe and generally well-tolerated at all doses
- No serious adverse events and no discontinuations
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry that included hepatic and renal
- Benign rash in some MAD subjects that was self-limited and resolved in 4 to 8 days post-onset
  - 2 subjects in 50 mg cohort, 7 subjects in 100 mg cohort

## PK/PD

- Linear, dose-proportional exposure
- Dose-related suppression of alternative pathway complement functional activity
- > 95% inhibition of alternative pathway in AP Wieslab® assay at 100 mg Q12hr through 7 days of dosing

## Program Advancing to Part 3 of Trial, PoC in PNH Patients

- Will evaluate both poor responders and treatment naïve PNH patients
- Data from PNH PoC expected 1H 2020