J.P. Morgan 36th Annual Healthcare Conference

January 10, 2018

Forward Looking Statements

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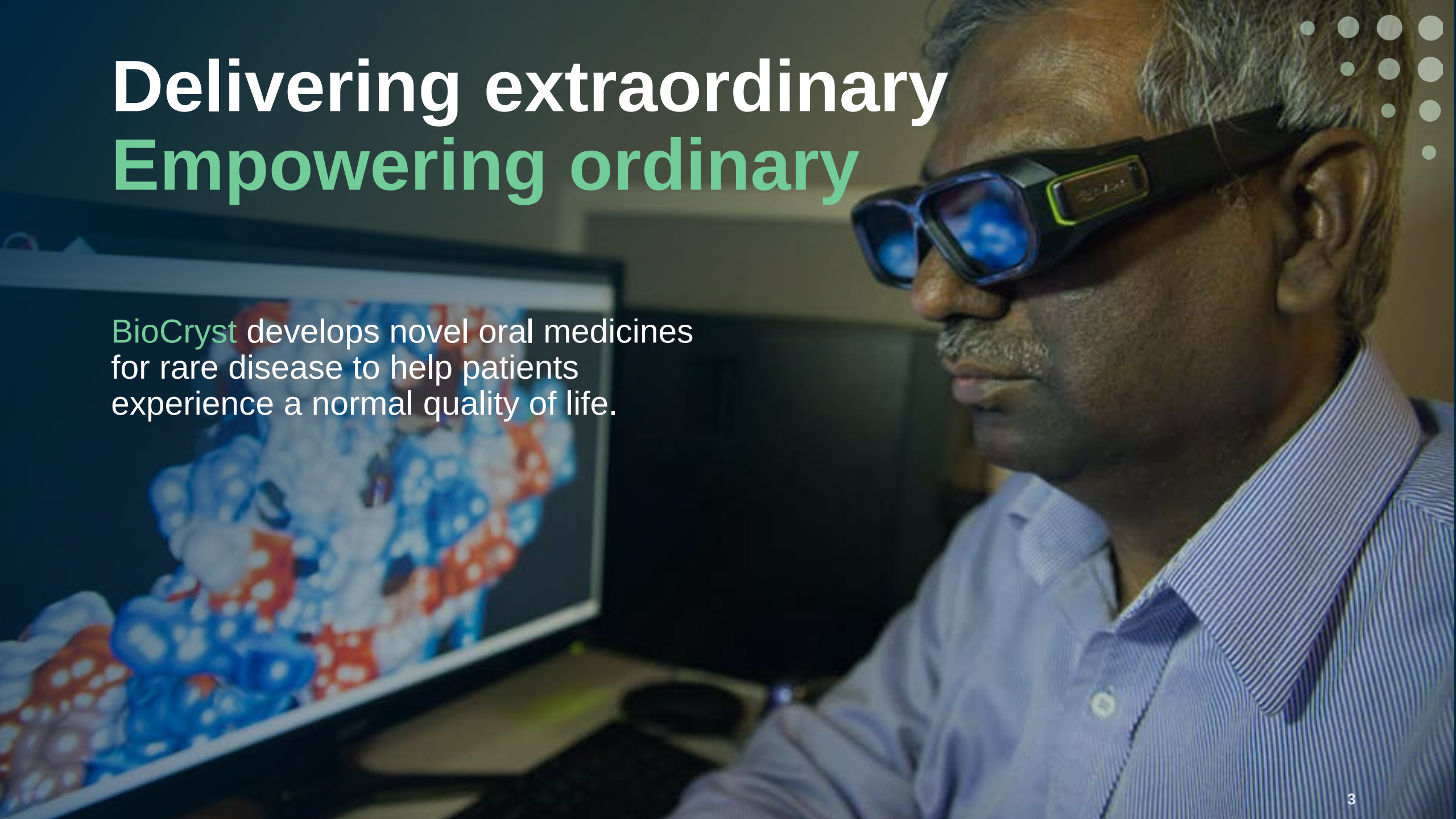
Delivering extraordinary
Empowering ordinary



BioCryst develops novel oral medicines for rare disease to help patients experience a normal quality of life.

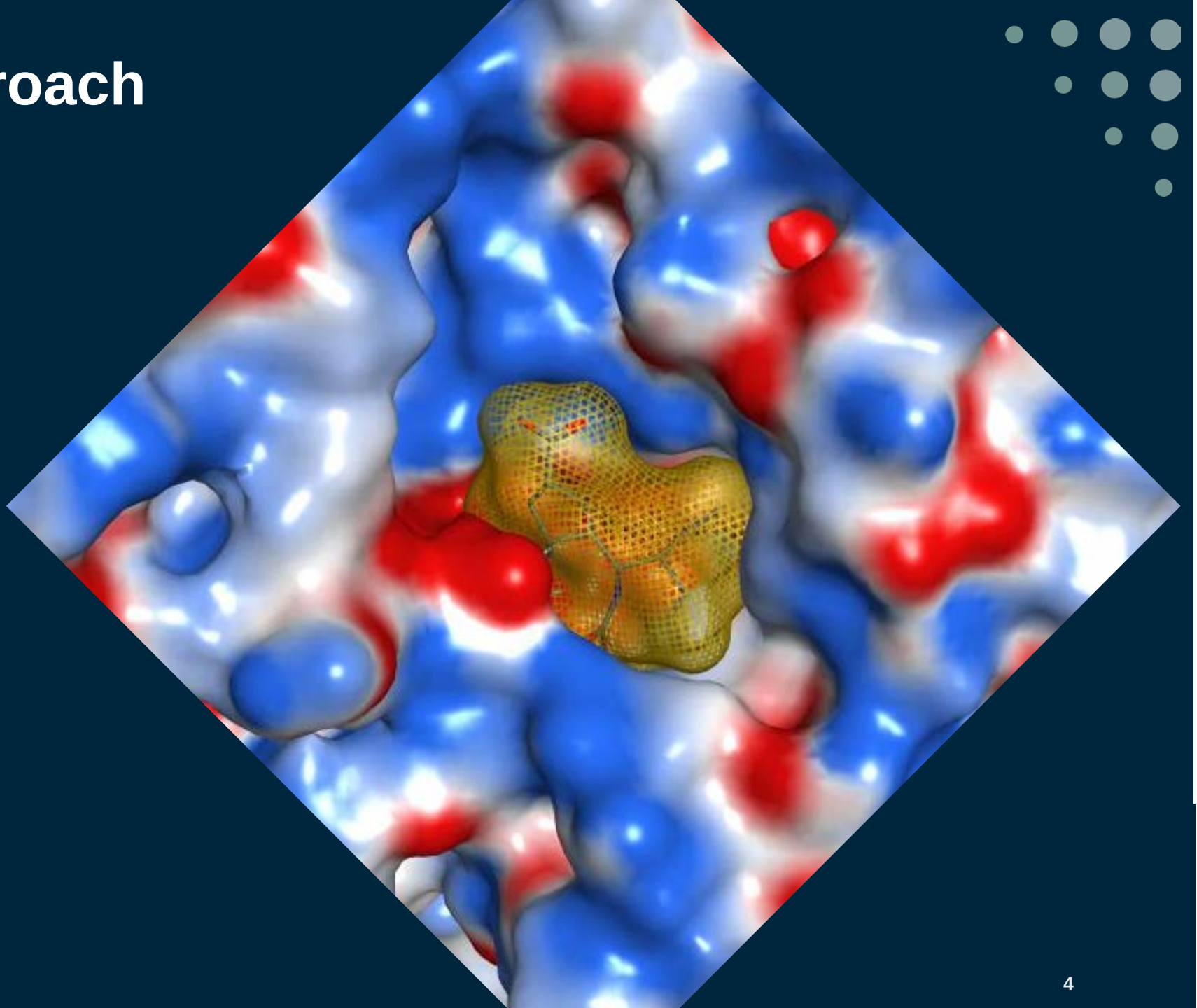
Our Unique Approach

STRUCTURE-GUIDED DRUG DESIGN

State-of-the-art structure-guided drug design to efficiently discover and develop new therapeutic candidates

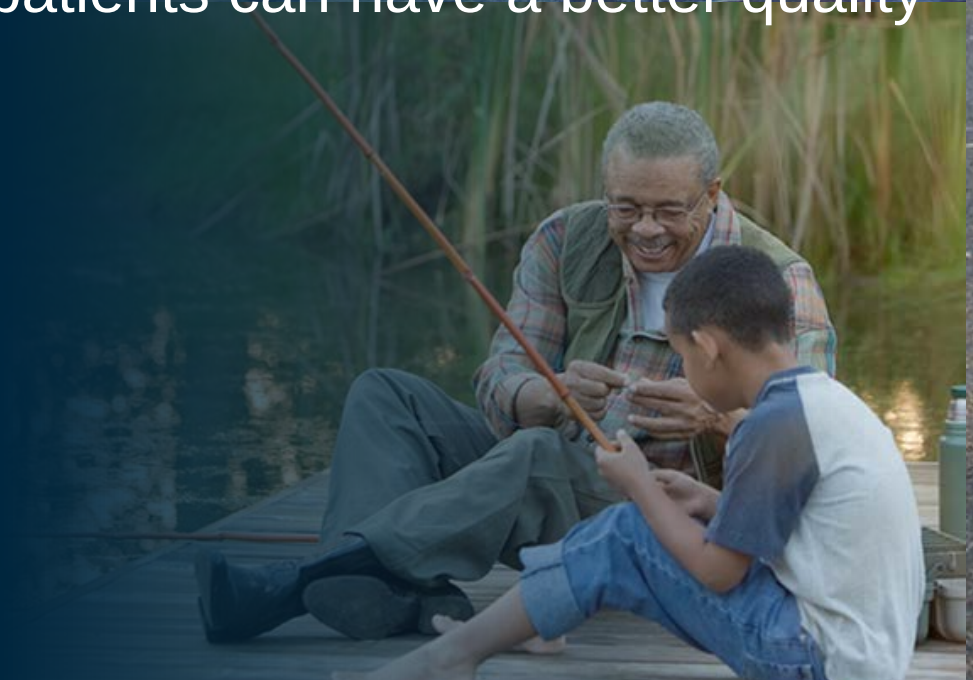
X-RAY CRYSTALLOGRAPHY

X-ray crystallography to determine the structures of active sites of targeted enzymes



Driven to Empower
Ordinary Living

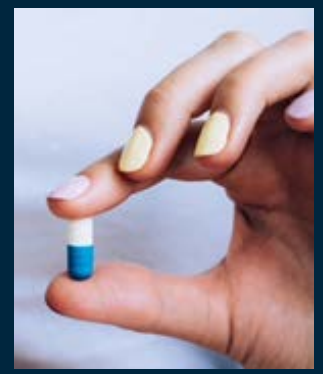
We’re committed to delivering extraordinary medicines so patients can have a better quality of life.



A New Era of Hereditary Angioedema Treatment

ANNUAL TREATMENT

ONCE A DAY



Unpredictable, debilitating, potentially life-threatening swelling attacks

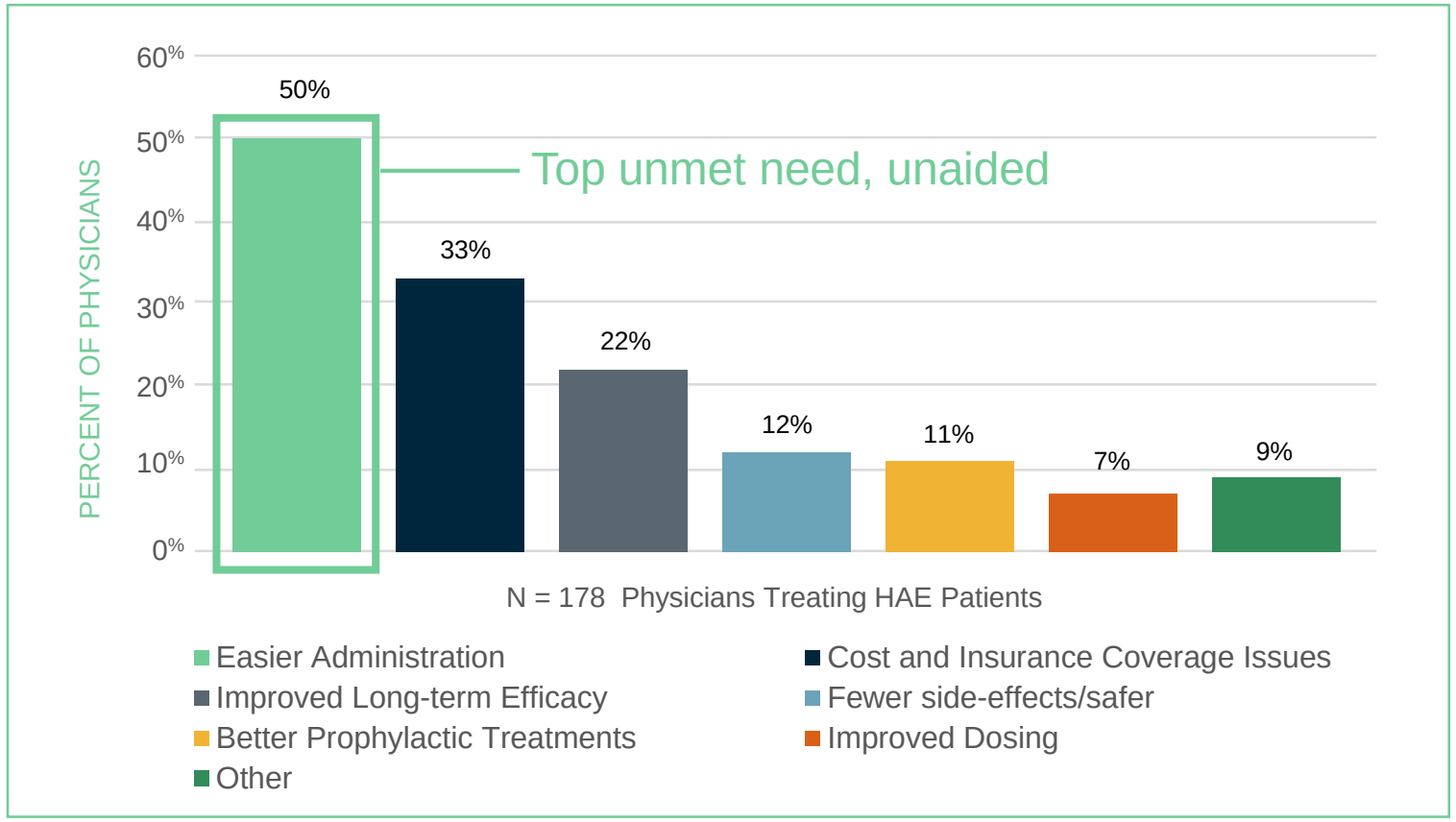
1 in 50,000 people affected worldwide

$2 Billion projected global market opportunity

BCX7353 is an oral once daily selective inhibitor of plasma kallikrein currently in Phase 3

Physicians and Patients Agree Ease of Administration is a High Unmet Need that will Drive Treatment Choice

Physician Unmet Needs in HAE Treatments



Public Meeting on Patient-Focused Drug Development for Hereditary Angioedema

- September 25, 2017

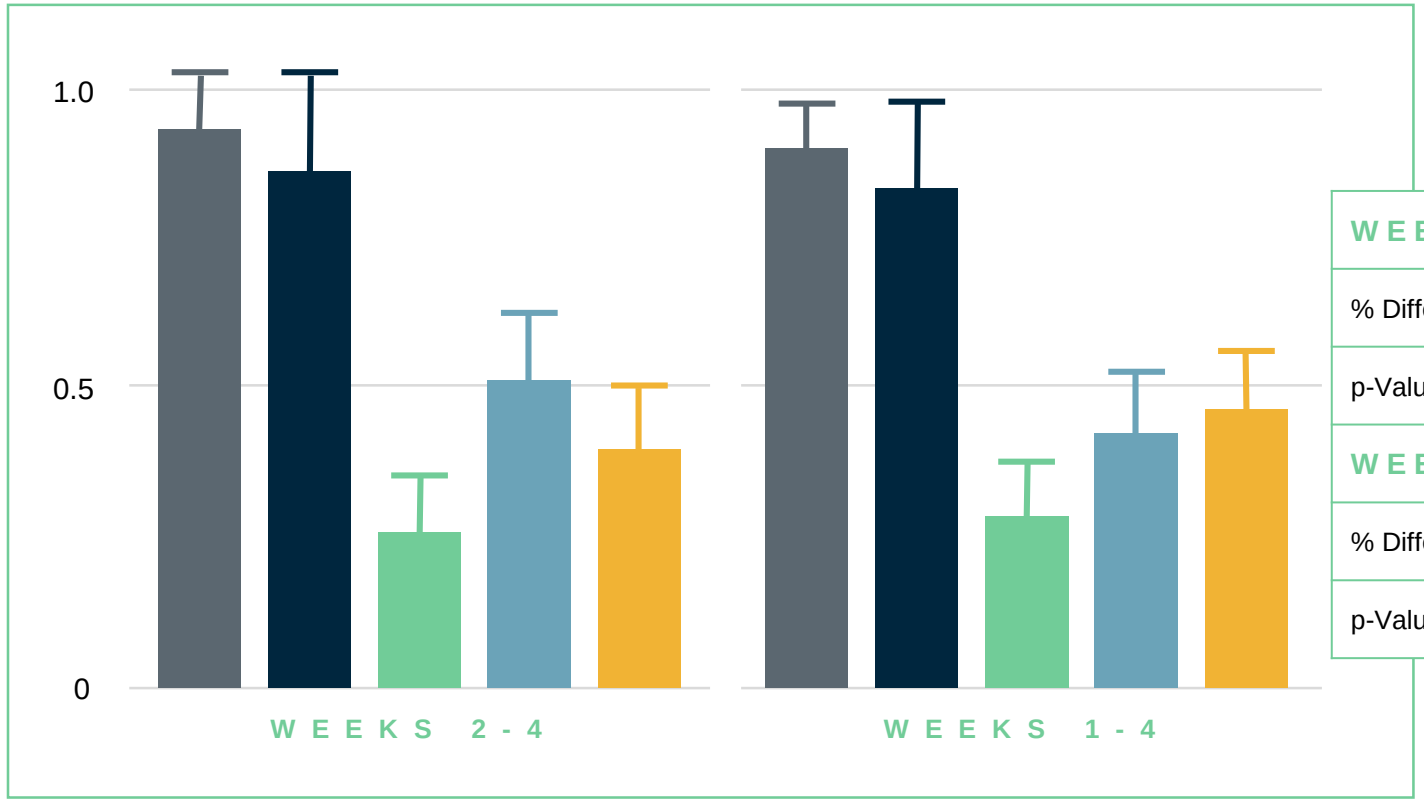
HAE Patients note **'method of administration'** as most important factor driving treatment choice; over access/cost, dose and side effect profile[1]

FDA U.S. FOOD & DRUG ADMINISTRATION

Source: BCRX proprietary market research study, 3Q17; 1) RBC Capital Markets 'FDA Patient Event Highlights HAE Unmet Need, Challenges, Opportunities, 9/25/17

APeX-1: Overall Angioedema Attack Rate per Week, PP Population, Weeks 2-4 and 1-4

Attack Rate: LS Mean Attacks/Week



	62.5mg	125mg	250mg	350mg
WEEKS 2-4				
% Difference, Active-PBO	-7%	-73%	-46%	-58%
p-Value	0.715	<0.001	0.006	<0.001
WEEKS 1-4				
% Difference, Active-PBO	-6%	-69%	-53%	-48%
p-Value	0.747	<0.001	<0.001	<0.001

Placebo BCX7353 62.5mg QD BCX7353 125mg QD BCX7353 250mg QD BCX7353 350mg QD

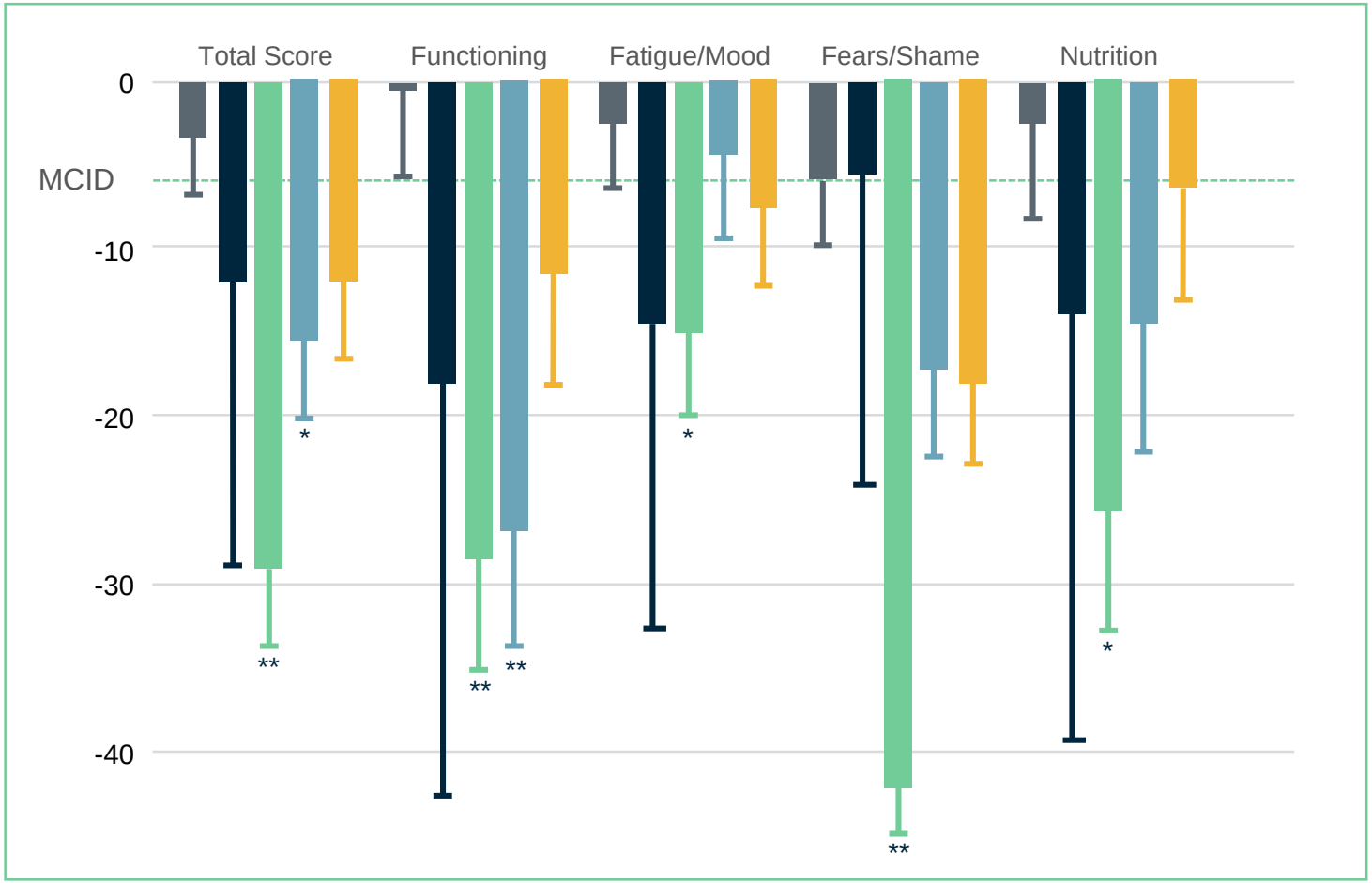
APeX-1: 125 mg Dose Provided Consistent Reductions in Attack Rate

Analysis	n	LS mean[1] Attacks per Week		Difference vs Placebo	Percentage Reduction vs Placebo	p-Value vs Placebo
		BCX7353 125 mg	Placebo			
Confirmed attacks, Weeks 2-4 PP population	13	0.248	0.932	-0.684	73%	<0.001
Confirmed attacks, Weeks 2-4 ITT population	14	0.249	0.937	-0.688	73%	<0.001
Confirmed attacks, Weeks 1-4 PP population	13	0.278	0.895	-0.617	69%	<0.001
Confirmed attacks, Weeks 1-4 ITT population	14	0.270	0.890	-0.619	70%	<0.001
Confirmed attacks requiring treatment, Weeks 2-4 PP population	13	0.221	0.807	-0.585	73%	<0.001
Confirmed attacks requiring treatment, Weeks 2-4 ITT population	14	0.224	0.771	-0.546	71%	0.002
Confirmed attacks requiring treatment, Weeks 1-4 PP population	13	0.221	0.788	-0.567	72%	<0.001
Confirmed attacks requiring treatment, Weeks 1-4 ITT population	14	0.217	0.753	-0.536	71%	<0.001

[1] Least squares mean calculated using an ANCOVA model with qualifying attack rate as covariate

APeX-1: Angioedema Quality of Life (AE-QoL): LS Mean Change from BL at Day 29, PP

QOL Score Improved



Difference in adjusted least square means are shown (Active treatment minus Placebo). ANCOVA Model includes terms of treatment and adjusted qualifying attack rate. Reductions (negative changes from BL) represent improvement in quality of life scores. MCID, minimum clinically important difference, -6 points (*Weller, K. 2016. Allergy 71(8): 1203-1209.)* BCX7353 dose level compared with placebo

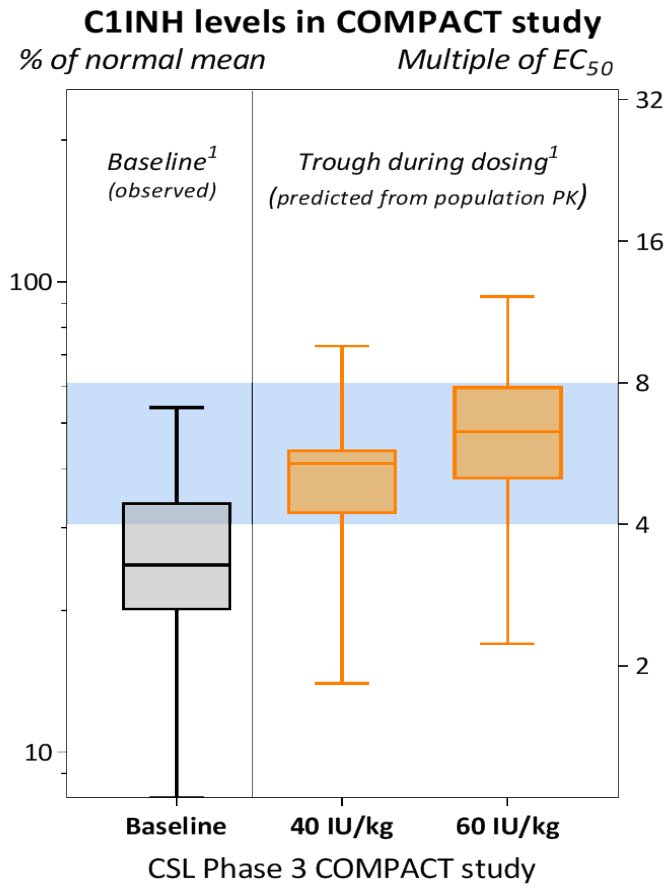
APeX-1: Treatment-Emergent Adverse Event Summary

Category	BCX7353				Placebo
	62.5 mg N = 7	125 mg N = 14	250 mg N = 14	350 mg N = 18	N = 22
Subjects with any TEAE[1], n (%)	4 (57)	7 (50)	11 (79)	14 (78)	15 (68.2)
Subjects with any Serious AE, n (%)	0	0	1 (7)[2]	0	0
Subjects with Drug-Related Grade 3/4 AE, n (%)	0	0	0	1 (6)	0
Subjects with AE Leading to D/C from Study Drug, n (%)	0	0	0	3 (17)	0
Non-drug-related, n (%)	0	0	0	1 (6)[3]	0
Drug-related, n (%)	0	0	0	2 (11)[4,5]	0

[1] TEAE- treatment-emergent adverse event.
[2] GI infection- investigator assessed as unrelated to study drug. Abdominal symptoms similar to several previous non-HAE-attack episodes occurring over past 3 years resulting in severe vomiting and diarrhea. Pt presented to ER and hospitalized for IV fluids and antiemetics as a precaution. No HAE acute attack meds given. Recovered and discharged the following day. Missed 1 day of dosing due to event.
[3] Pre-existing liver disorder (improved from baseline, but persisting). Previously reported in 1st interim analysis.
[4] n=1 Gastroenteritis with liver disorder (both assessed as related) (elevated ALT 1.9x ULN, GGT 5.4x ULN and ALP 1.6x ULN, with normal AST and bilirubin). Previously reported in 1st interim analysis.
[5] n=1 Vomiting/abdominal cramps. Previously reported in 2nd interim analysis.

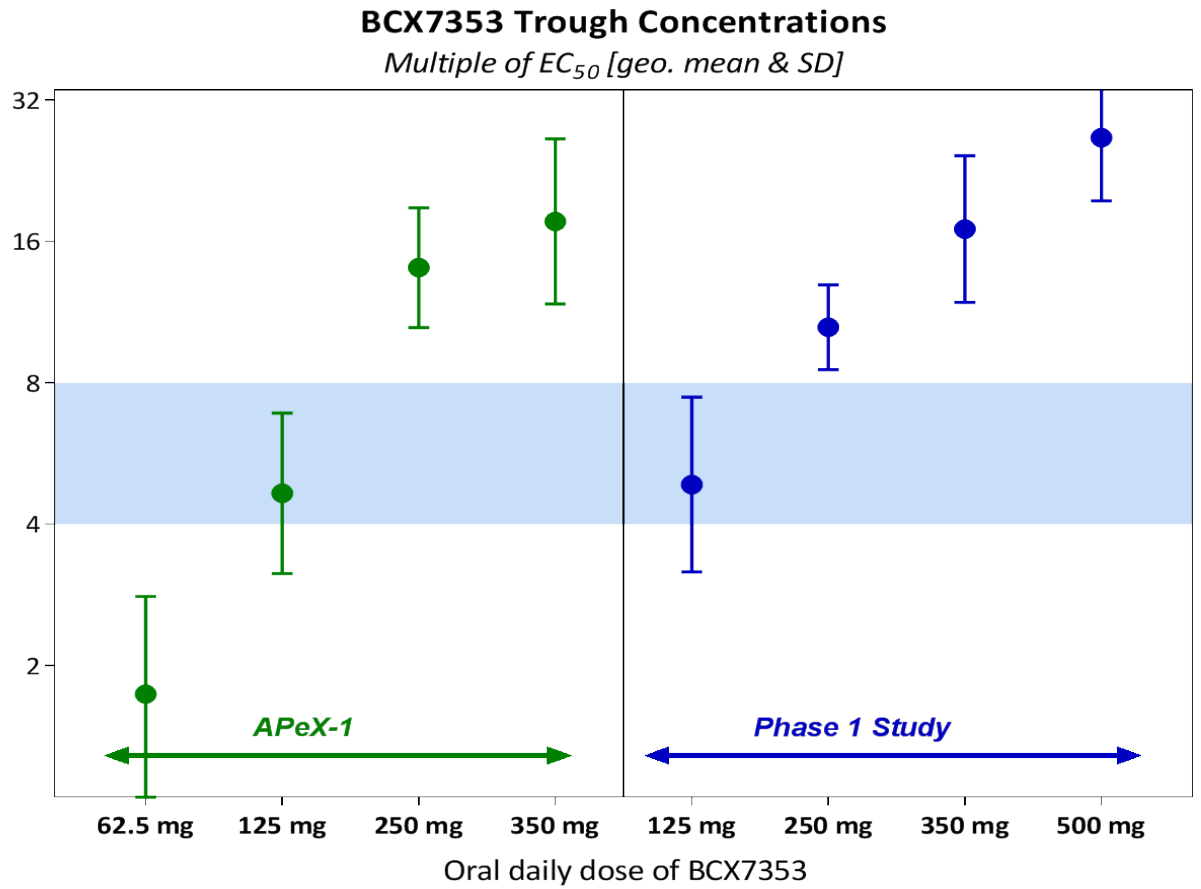
APeX-1: Exposure Comparisons of BCX7353 and SC C1INH

CSL-830 Phase 3 Study



C1INH levels at baseline and after SC dosing with CSL-830[1]

BCX7353 APeX-1 & Phase 1 Study



BCX7353 plasma concentrations at 24 hours post-dose

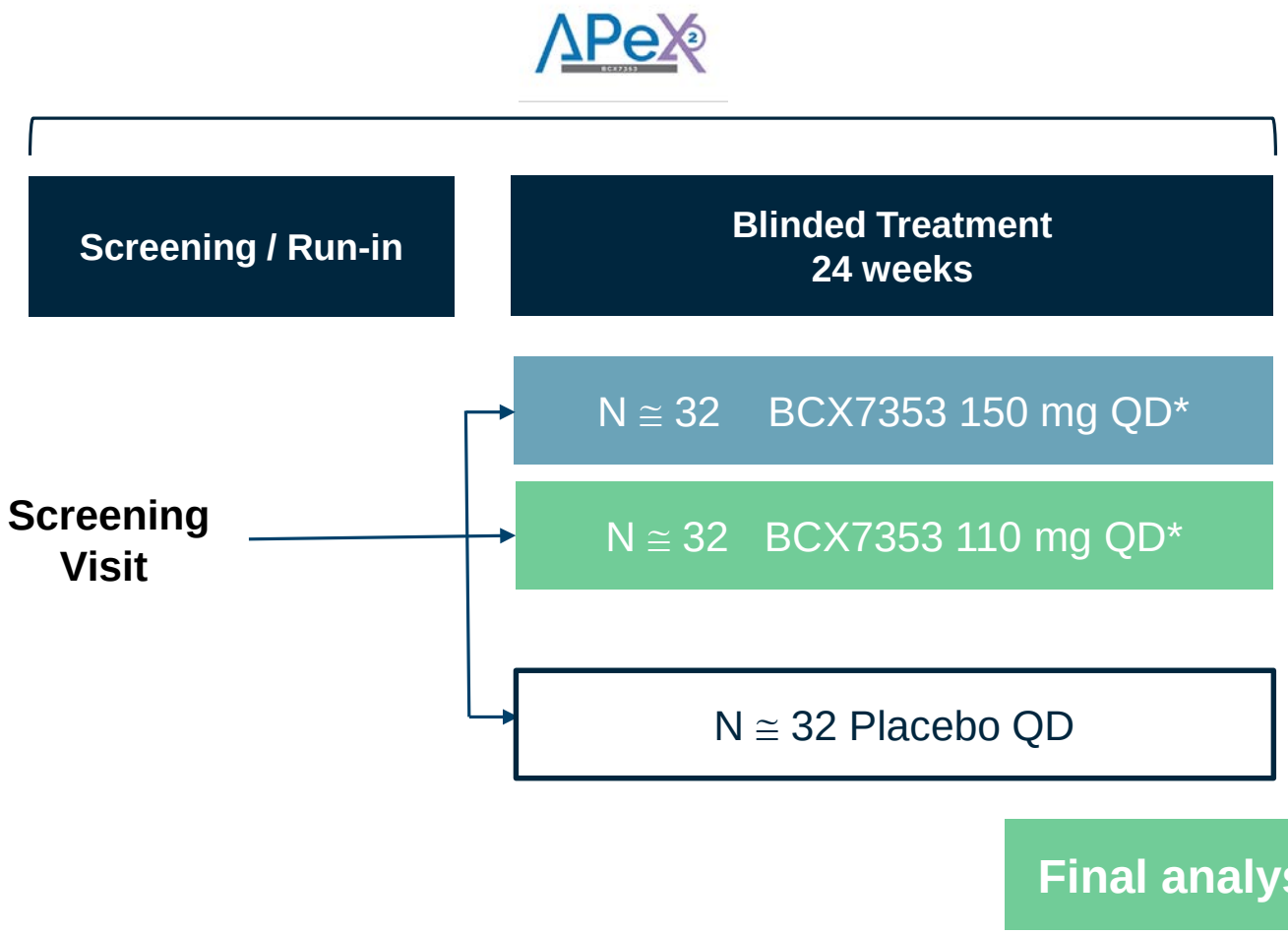
[1] Longhurst, H. et al. N Engl J Med 376, 1131-1140 (2017). Box plots represent median, 25th and 75th percentiles, minimum and maximum values. CSL-830 and BCX7353 data are from distinct clinical trials and no head to head study has been conducted.

Predictable PK Supports 175 mg as Second Dose in Phase 3

Dose, mg QD	% > 4 x EC_{50}		% > 6 x EC_{50}		% > 8 x EC_{50}	
	Predicted	Actual	Predicted	Actual	Predicted	Actual
62.5	--	*0*	--	*0*	--	*0*
125	70	*64*	38	*43*	17	*0*
175	93		80		58	
200	97		88		73	
225	98		93		83	
250	100	*100*	97	*100*	93	*100*

- **Given the predictable PK of BCX7353, simulations are helpful in selecting an intermediate dose of BCX7353 to study that is between 125 mg and 250 mg.**
- **These simulations suggest a relatively small increase in dose above 125 mg should achieve a significant increase in the proportion of subjects achieving trough levels above the therapeutic target.**
- **These simulations suggest 175 mg dose should maintain trough drug levels > 4 x EC_{50} in > 90% of patients.**
- **Doses ≥ 200 mg offer little additional increment in proportions achieving target level.**

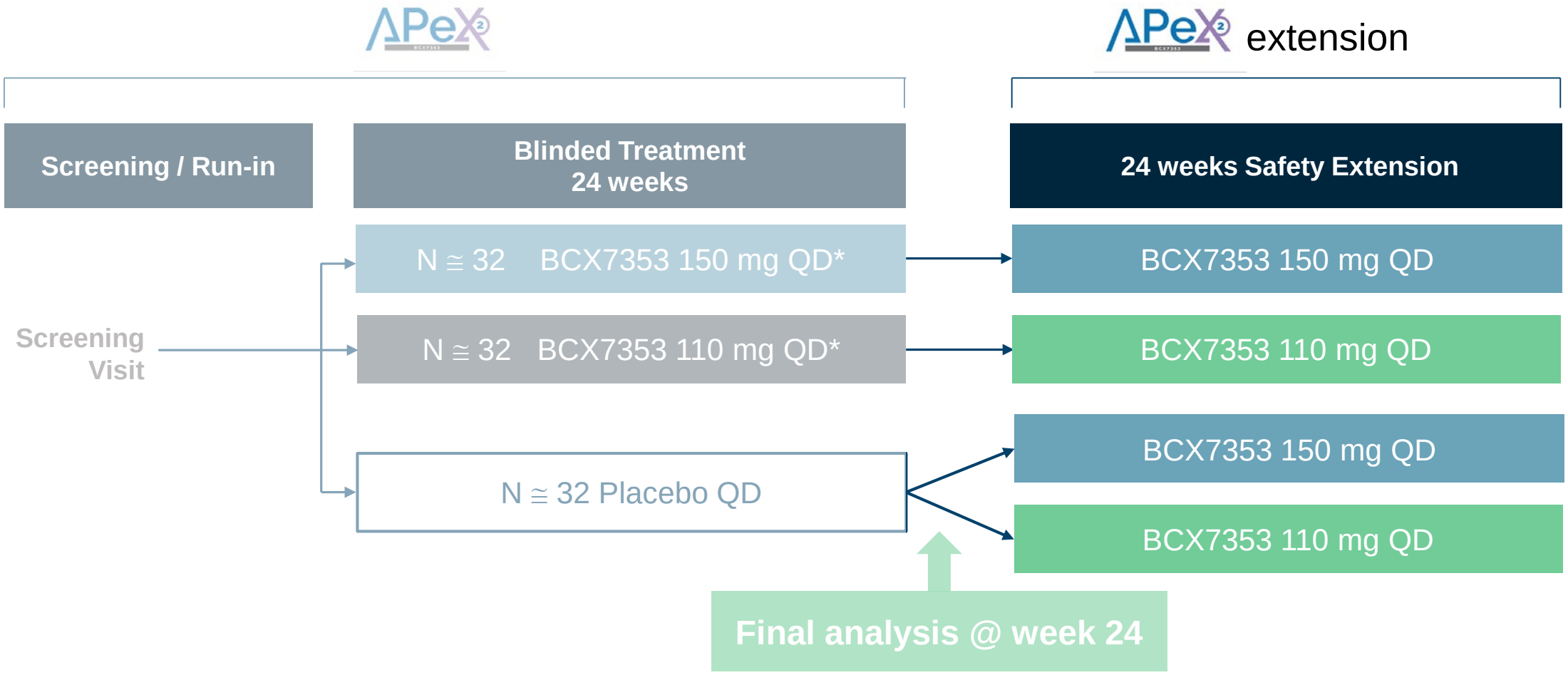
APeX-2: Phase 3 Trial Design



- Primary endpoint at Week 24:
  - Rate of Investigator-confirmed HAE attacks through entire treatment period
- Study powered at >90% to detect a ≥50% reduction in attack rate over placebo

*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt:
150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt

APeX-2: Phase 3 Trial Design – Safety Extension



*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt:
150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt

APeX-S: Long-term Safety Study Design

48 Weeks Treatment

N ≅ 80 BCX7353 150 mg QD

N ≅ 80 BCX7353 110 mg QD

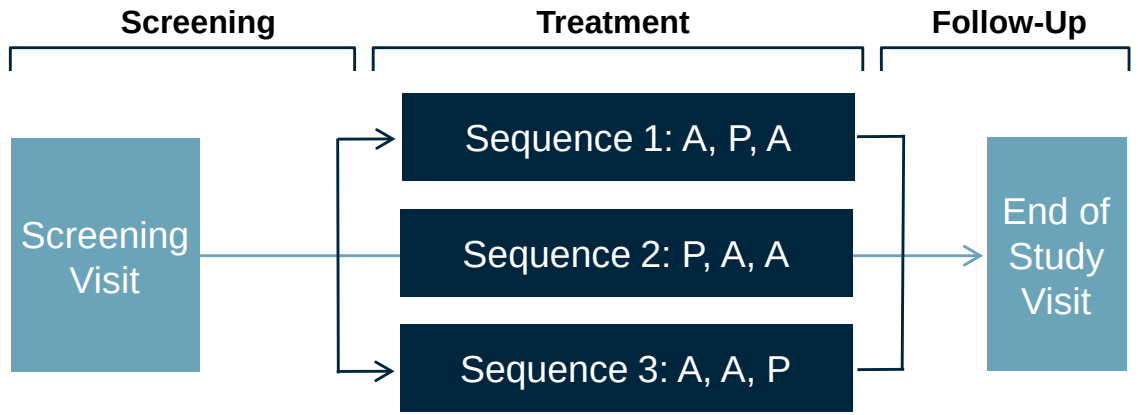
Analyses as needed for regulatory submissions

- Endpoints:
  - Long term safety of BCX7353
  - Durability of response
  - Quality of Life
- APeX-1 subjects eligible

- Safety database:
  - Up to 100 subjects at each dose level
  - Combination of APeX-2 extension and APeX-S

*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt:
150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt

ZENITH-1: Phase 2 Trial Design – Oral Liquid



Part 1: BCX7353 750 mg single doses n = 12 - 36	+	Part 2: BCX7353 500 mg single doses n = 12	+	Part 3: BCX7353 250 mg single doses n = 12	=	Total n = 36 - 60

Primary Efficacy Endpoint:

- Proportion of subjects with either improved or stable composite visual analog scale (VAS) score at 4 hours post-dose.

Fibrodysplasia Ossificans Progressiva (FOP)

Devastating Disease; No Treatments Available

Rare disease that **affects approximately 1 in 2 million people** worldwide

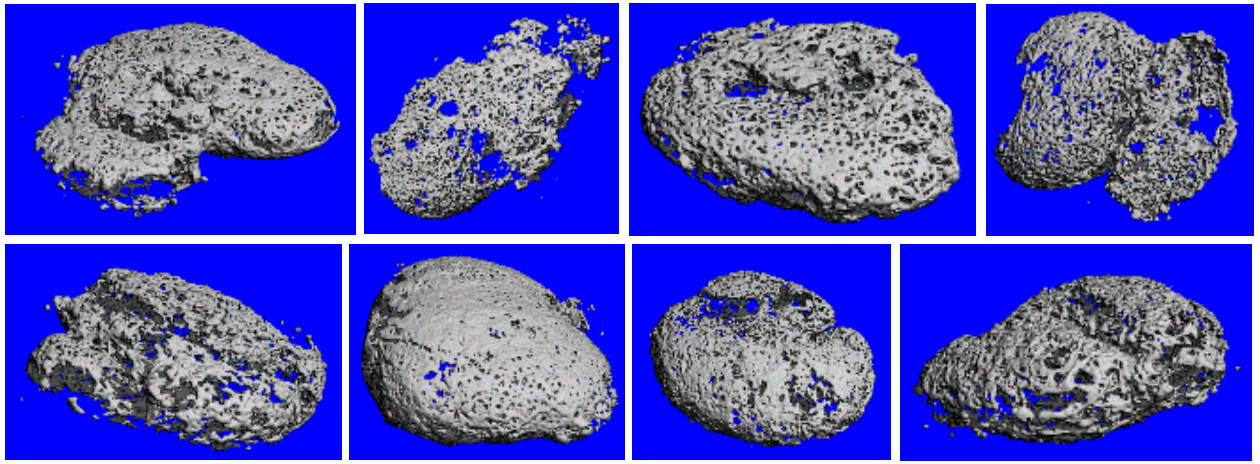
Irregular formation of bone or ossification in muscles, tendons or soft tissue

Currently no approved treatments for FOP

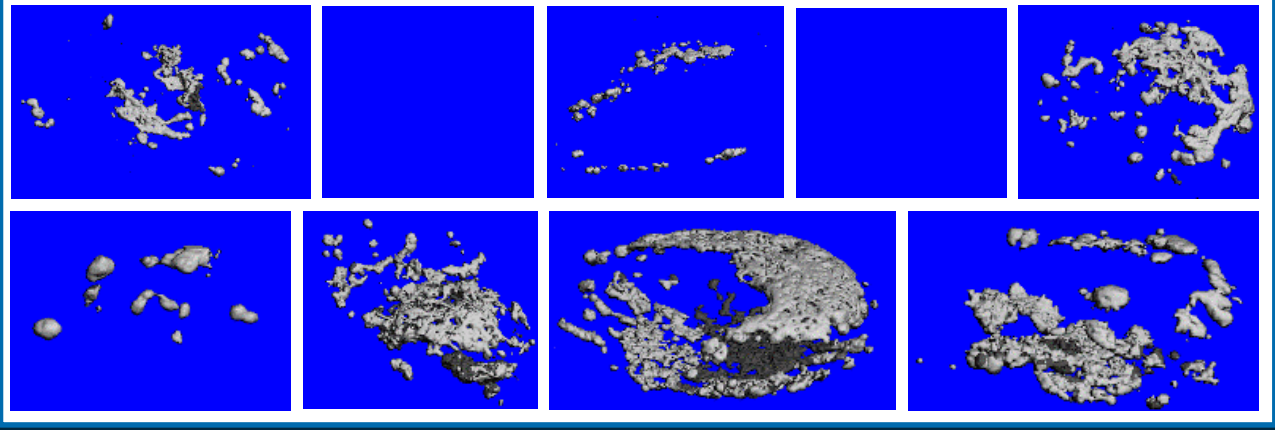
Results in loss of function, deformities and **a severely disabling condition**

Advancing ALK2 Inhibitor Program for FOP

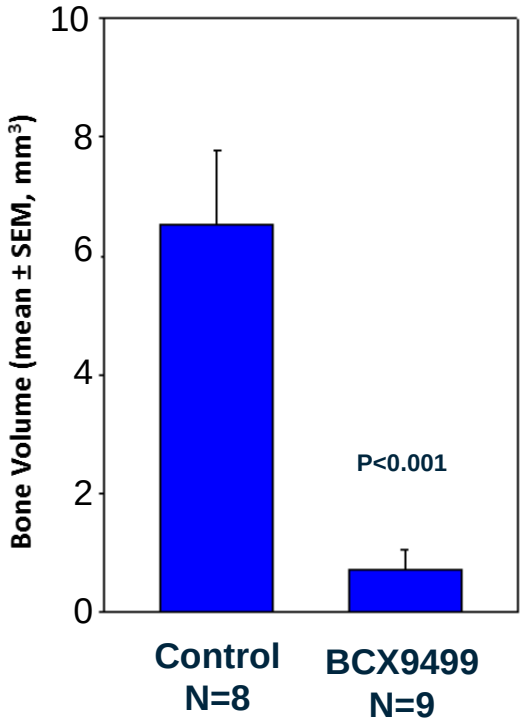
Control Vehicle



BCX9499 Treated 30mg/Kg BID



89% Reduction in Bone Formation Compared to Control Vehicle



Micro computed tomography scans of heterotopic bone induced by subcutaneous implantation of matrigel mixed with rhBMP9 and cardiotoxin.

BioCryst's Robust Pipeline

	Lead Optimization	Pre-clinical	Phase 1	Phase 2	Phase 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral Capsule (Prophylactic HAE)							
BCX7353 – Oral Liquid (Acute HAE)							
Second generation kallikrein inhibitors (HAE & Other Indications)							
BCX9250 & BCX9499 (FOP)							
Rare diseases							
SUPPORTING ASSETS: Externally funded, potential for capital infusions							
RAPIVAB® (peramivir injection)*							
Galidesivir (Broad spectrum antiviral) I.V.							

*Licensed to Seqirus, Shionogi and Green Cross

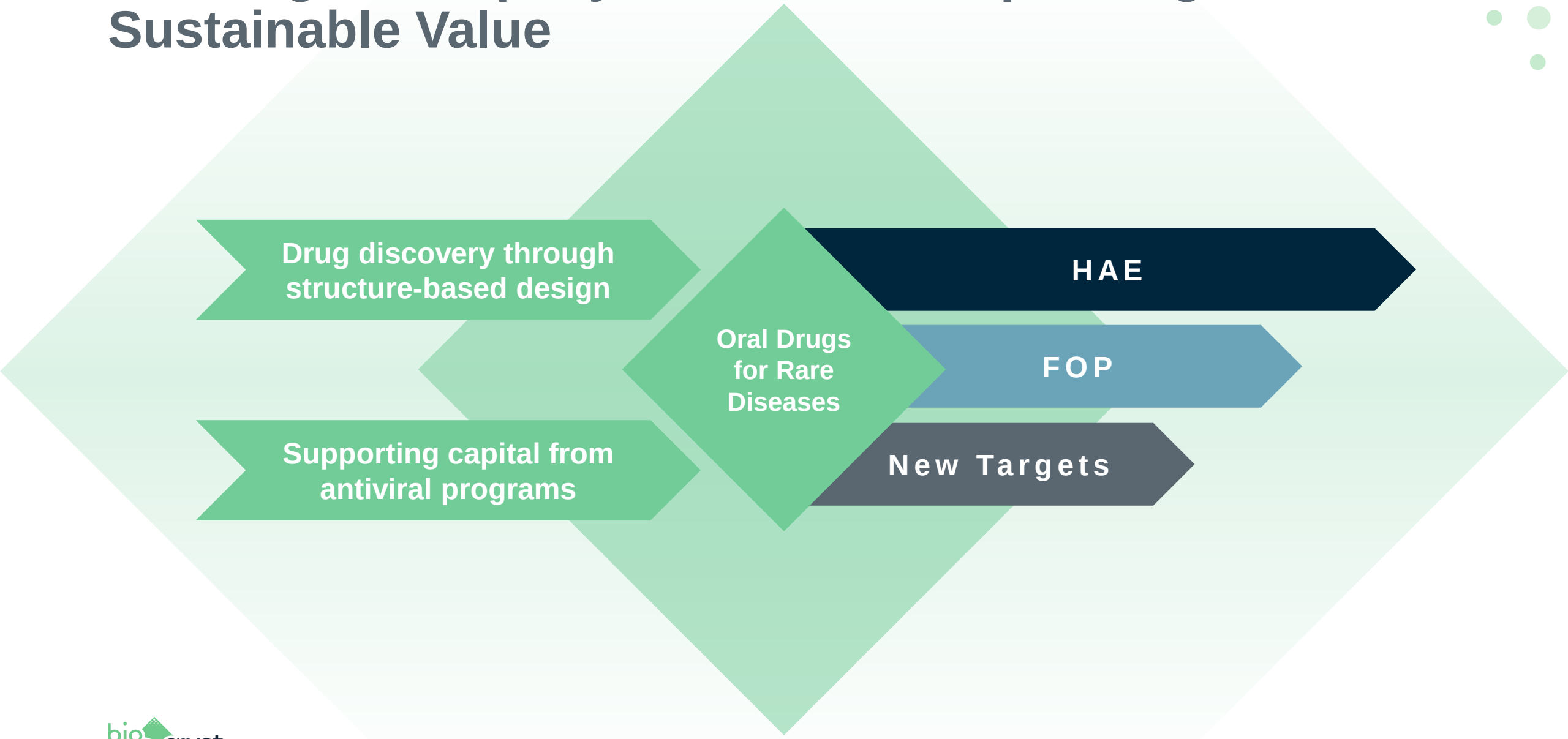
Cash Position & 2017 Guidance (In millions)

Cash & investments at December 31, 2016	$65
Cash & investments at September 30, 2017	**$169**
Senior Credit Facility	$23
FY 2017 GUIDANCE	
Operating cash utilization	**$30 – 50@**
Operating expenses#	**$53 – 73@**

Excludes equity-based compensation.

@ We currently forecast our actual results to be in the upper-half of our 2017 Guidance.

Building A Company to Generate Expanding and Sustainable Value



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