

J.P. Morgan Healthcare Conference

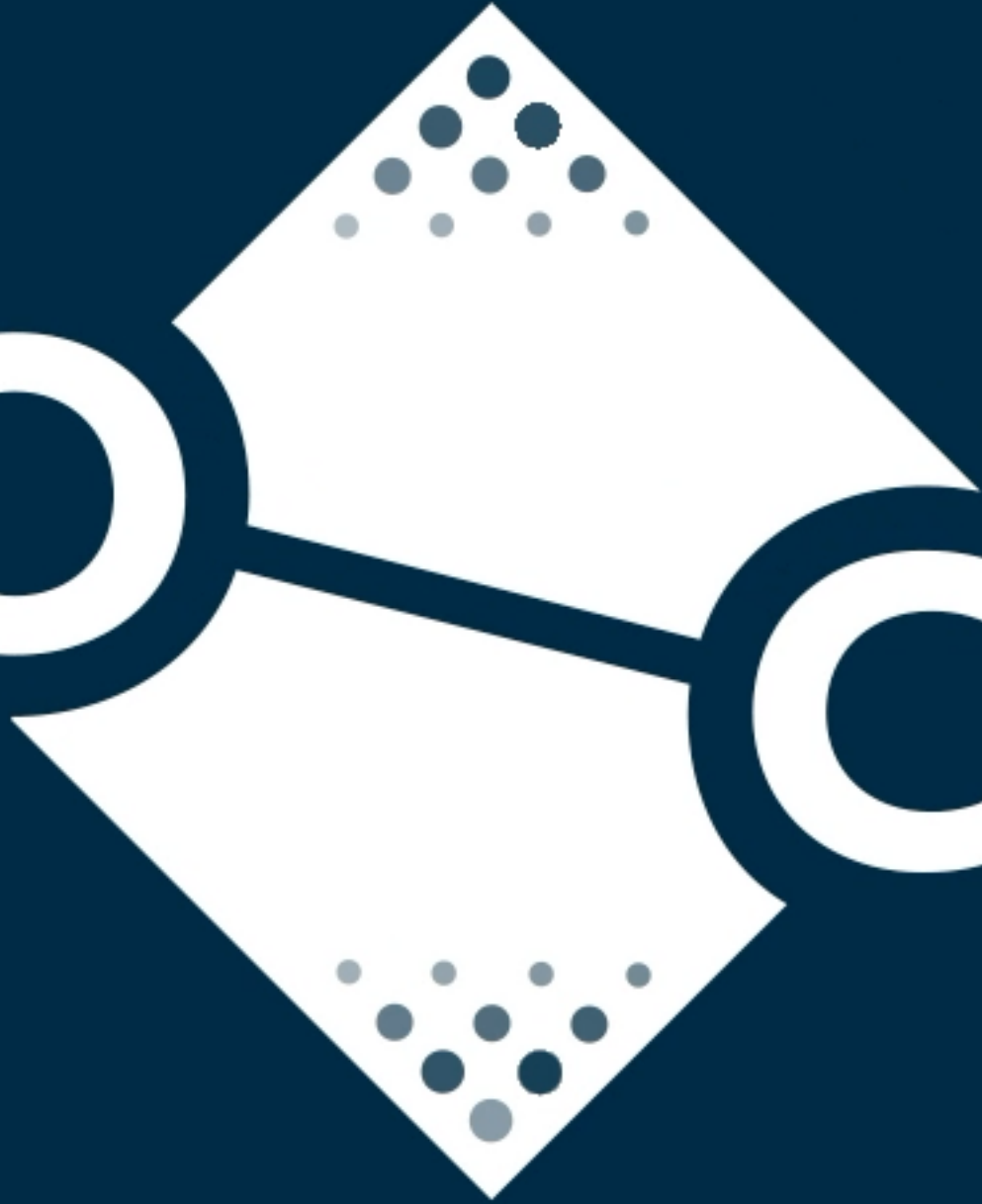
January 13, 2021

BioCryst Pharmaceuticals

Jon Stonehouse, **CEO**

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 ir.biocryst.com/financial-information/sec-filings

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This is big.

HAEA Patient Summit

September, 2013

HAE attacks are chronic and unpredictable

HAE attacks increase anxiety and depression for patients

48% of HAE patients experiencing >13 attacks in
the past 6 months are unemployed

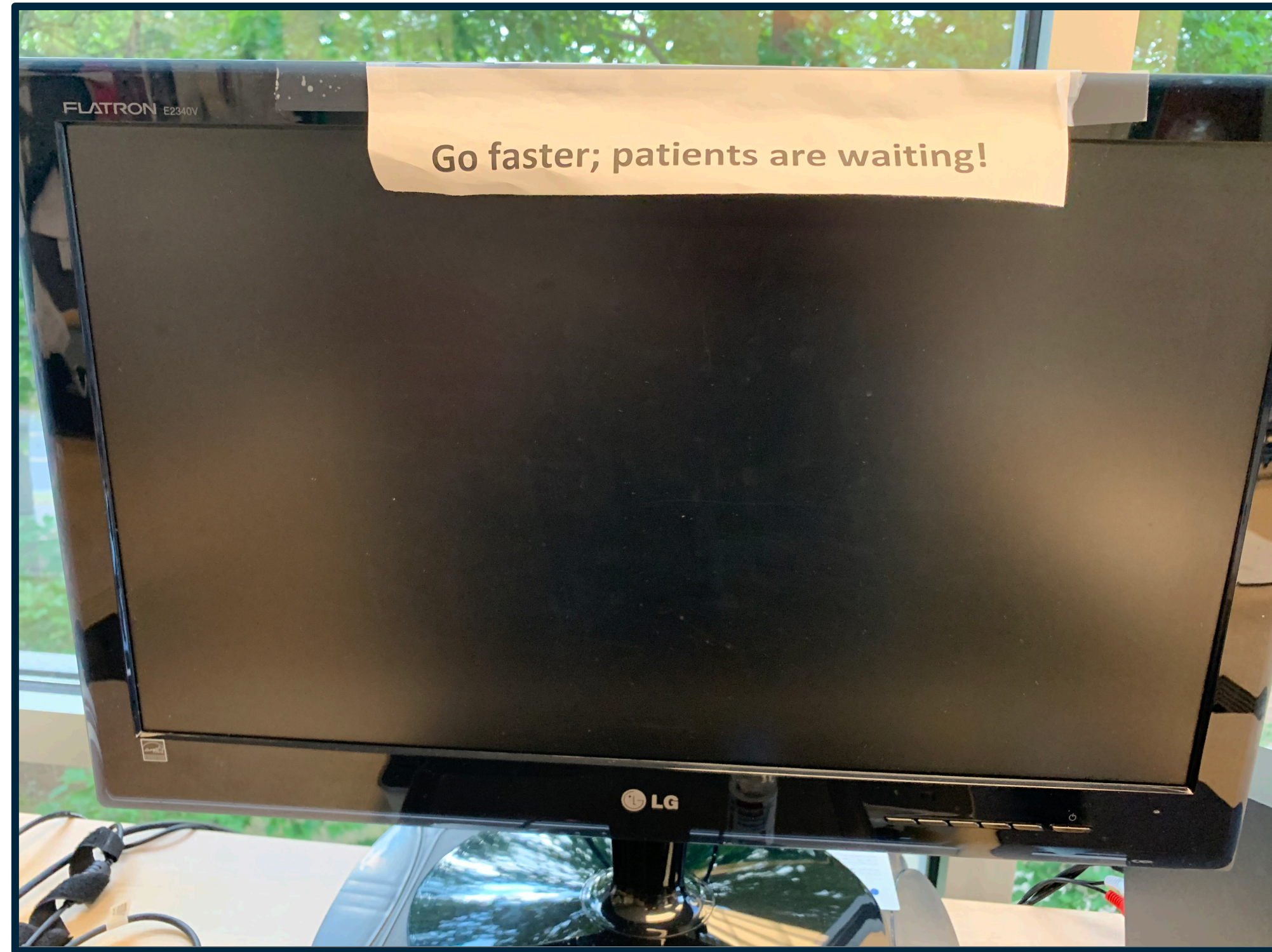
~50% of HAE patients have at least one laryngeal attack
in their lifetime

82% of HAE patients agreed a once-daily oral pill to prevent attacks would allow them to feel more freedom from their HAE

“ WHY ISN'T THERE A PILL FOR HAE? ”

“ GO FASTER; PATIENTS ARE WAITING. ”

This is big.



go fast..
patients
are waiting

For hereditary
angioedema (HAE),
This is big.



Capsule not actual size

INDICATION

ORLADEYO™ (berotralstat) is a plasma kallikrein inhibitor indicated for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adults and pediatric patients 12 years and older.

Limitations of use

The safety and effectiveness of ORLADEYO for the treatment of acute HAE attacks have not been established. ORLADEYO should not be used for the treatment of acute HAE attacks. Additional doses or dosages of ORLADEYO higher than 150 mg once daily are not recommended due to the potential for QT prolongation.

orladeyo™
(berotralstat) capsules 150 mg

bio  **cryst**™

Estimated 2,500 HAE patients in Japan

Granted Sakigake designation by the Japanese PMDA

ORLADEYO would be 1st approved prophylaxis treatment
for HAE in Japan

First HAE candidate to receive positive scientific opinion through the Early Access to Medicines Scheme (EAMS) in the UK

EU HAE prevalence estimated as 1 in 50,000

Promising Innovative Medicine (PIM) designation granted by
MHRA April 2018



NDC 72769-101-01

RX only

orladeyo[™]
(berotralstat) capsules 150 mg

Contains seven (7) 150 mg capsules
FOR ORAL USE ONLY



To open
Step 1: Press & hold button gently
Step 2: Pull out medication card

bio-cryst[™]

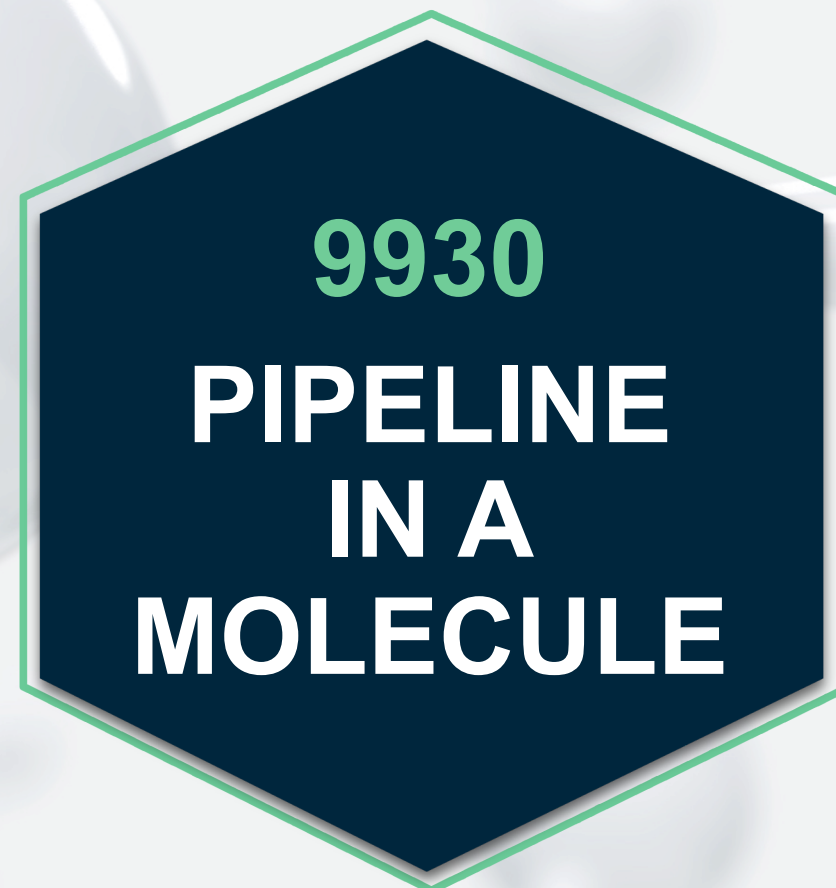
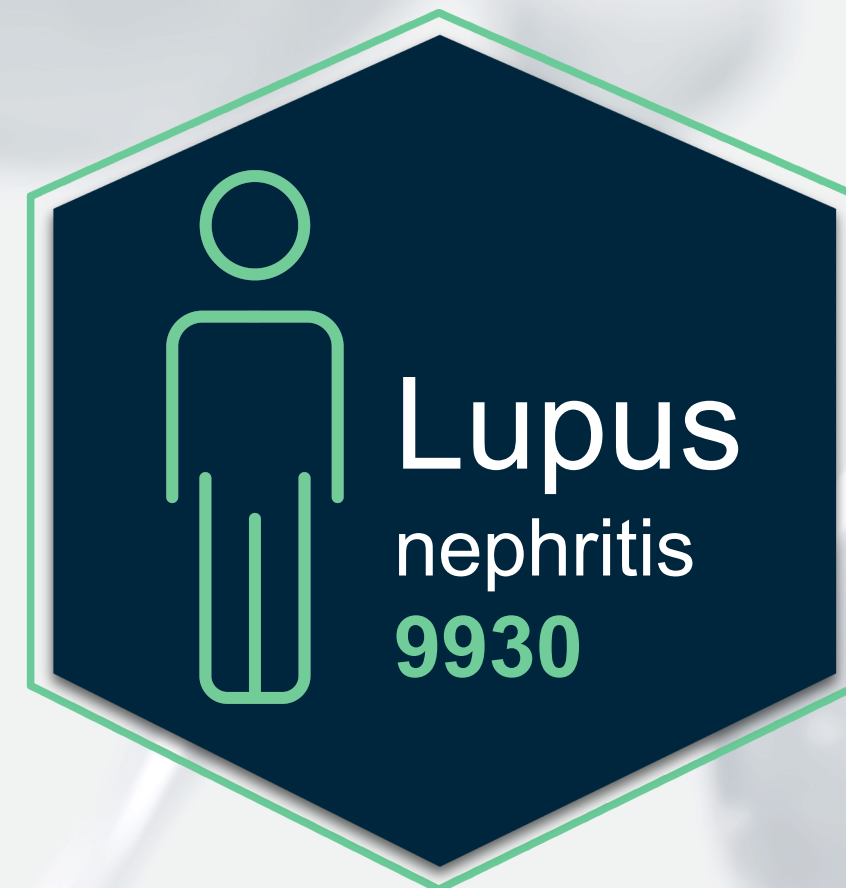
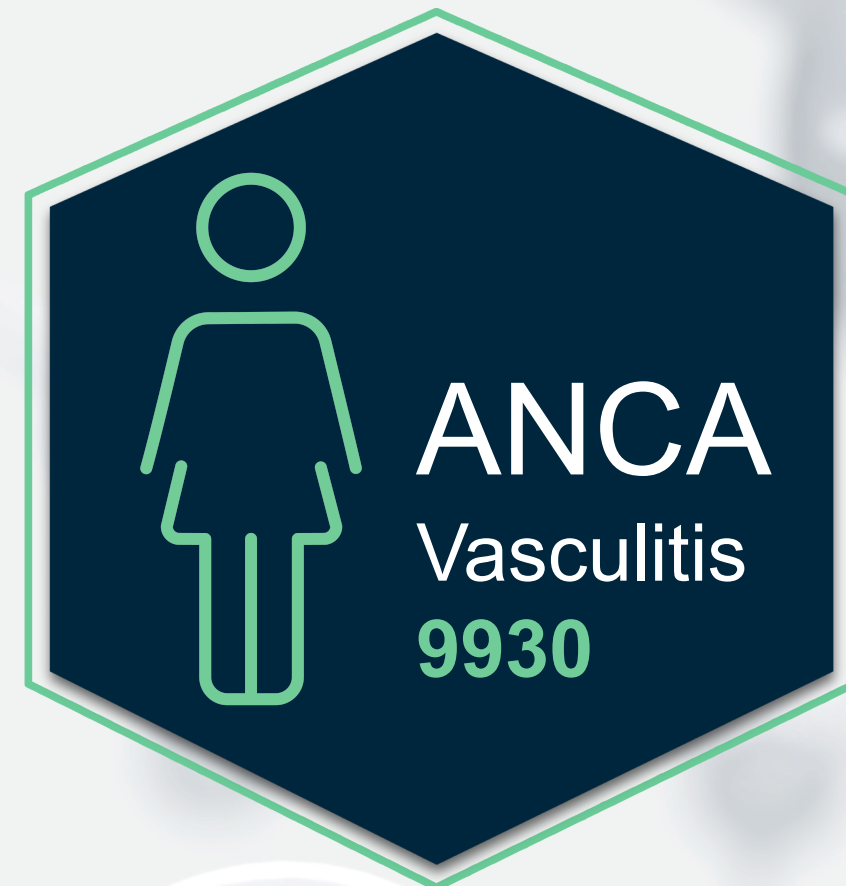
NDC 72769-101-01

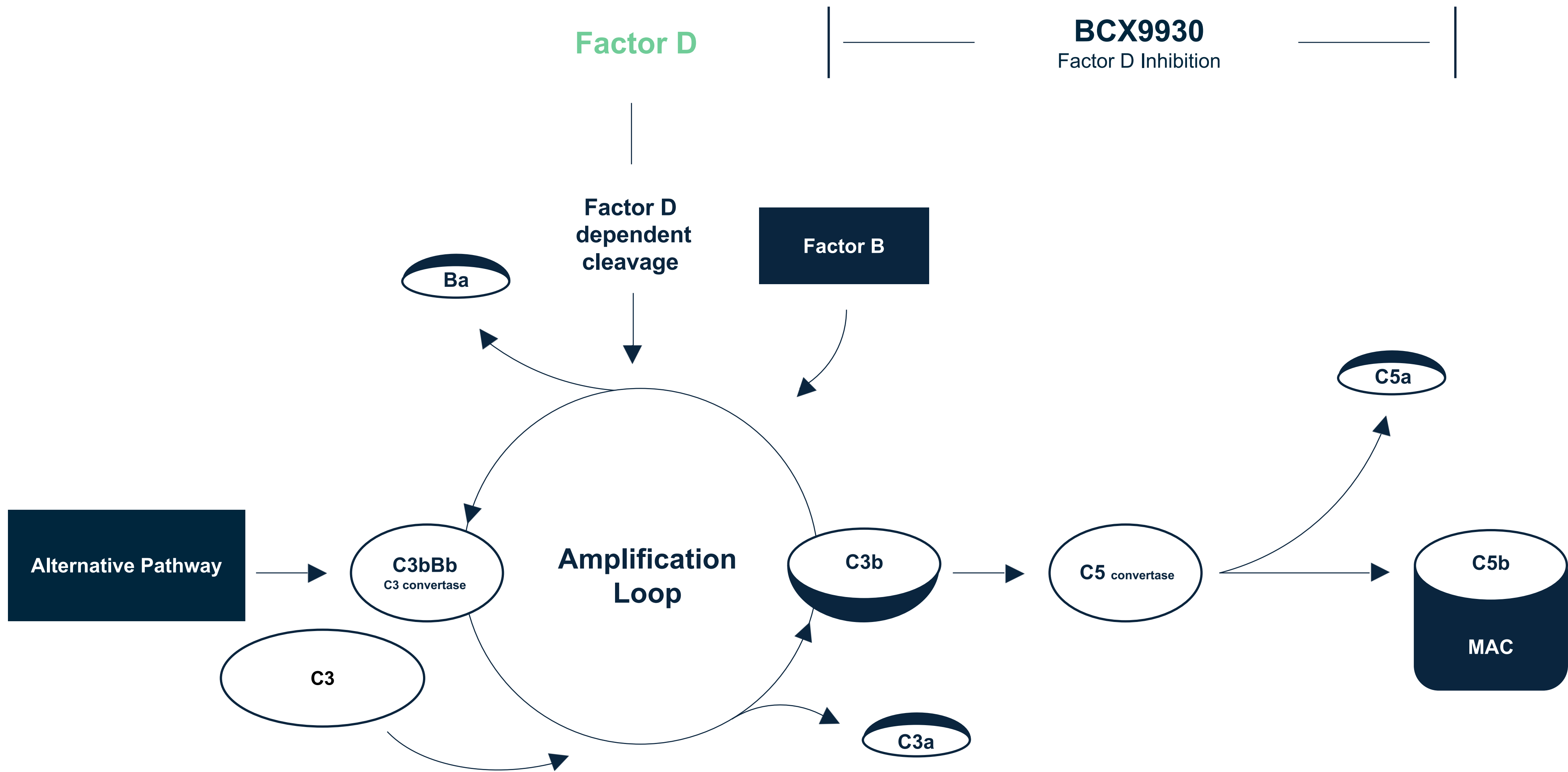
RX only

orladeyo[™]
(berotralstat) capsules 150 mg

FOR ORAL USE ONLY
28 capsules
Contains a 28-day supply
4 shellpacks each containing a 7-capsule blister card

bio-cryst[™]





Alternative Complement Pathway Activation Products in Urine and Kidneys of Patients with ANCA-Associated GN

Shen-Ju Gou, Jun Yuan, Chen Wang, Ming-Hui Zhao, and Min Chen

Clin J Am Soc Nephrol 8: 1884–1891, 2013. doi: 10.2215/CJN.02790313

Complement activation products in the circulation and urine of primary membranous nephropathy

Mu-fan Zhang^{1,2,3,4}, Jing Huang^{1,2,3,4}, Yi-miao Zhang^{1,2,3,4}, Zhen Qu^{1,2,3,4}, Xin Wang^{1,2,3,4}, Fang Wang^{1,2,3,4}, Li-qiang Meng^{1,2,3,4}, Xu-yang Cheng^{1,2,3,4}, Zhao Cui^{1,2,3,4*}, Gang Liu^{1,2,3,4} and Ming-hui Zhao^{1,2,3,4,5}

BMC Nephrology (2019) 20:313

The Emerging Role of Complement Proteins as a Target for Therapy of IgA Nephropathy

Dana V. Rizk^{1*}, Nicolas Maillard², Bruce A. Julian¹, Barbora Knoppova^{3,4}, Todd J. Green³, Jan Novak³ and Robert J. Wyatt^{5*}

Frontiers in Immunology | www.frontiersin.org

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March 2019 | Volume 10 | Article 504

CJASN[®] Clinical Journal of American Society of Nephrology

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*||

frontiers
in Immunology

REVIEW
published: 14 June 2019
doi: 10.3389/fimmu.2019.01157

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 Ricci¹, Luana Marano¹, Camilla Frieri¹, Fabiana Cacace¹, Michela Sica³, Austin Kulasekararaj^{3,4}, Rodrigo T. Calado⁵, Phillip Scheinberg⁶, 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



Complement Alternative Pathway's Activation in Patients With Lupus Nephritis

Di Song, PhD, Wei-yi Guo, PhD, Feng-mei Wang, PhD, Yong-zhe Li, PhD, Yan Song, MD, Feng Yu, MD and Ming-hui Zhao, MD, PhD

Am J Med Sci 2017;353(3):247–257

Seminars in
**Thrombosis
and Hemostasis**

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

Sanjeev Sethi, MD, PhD¹ Fernando C. Fervenza, MD, PhD²

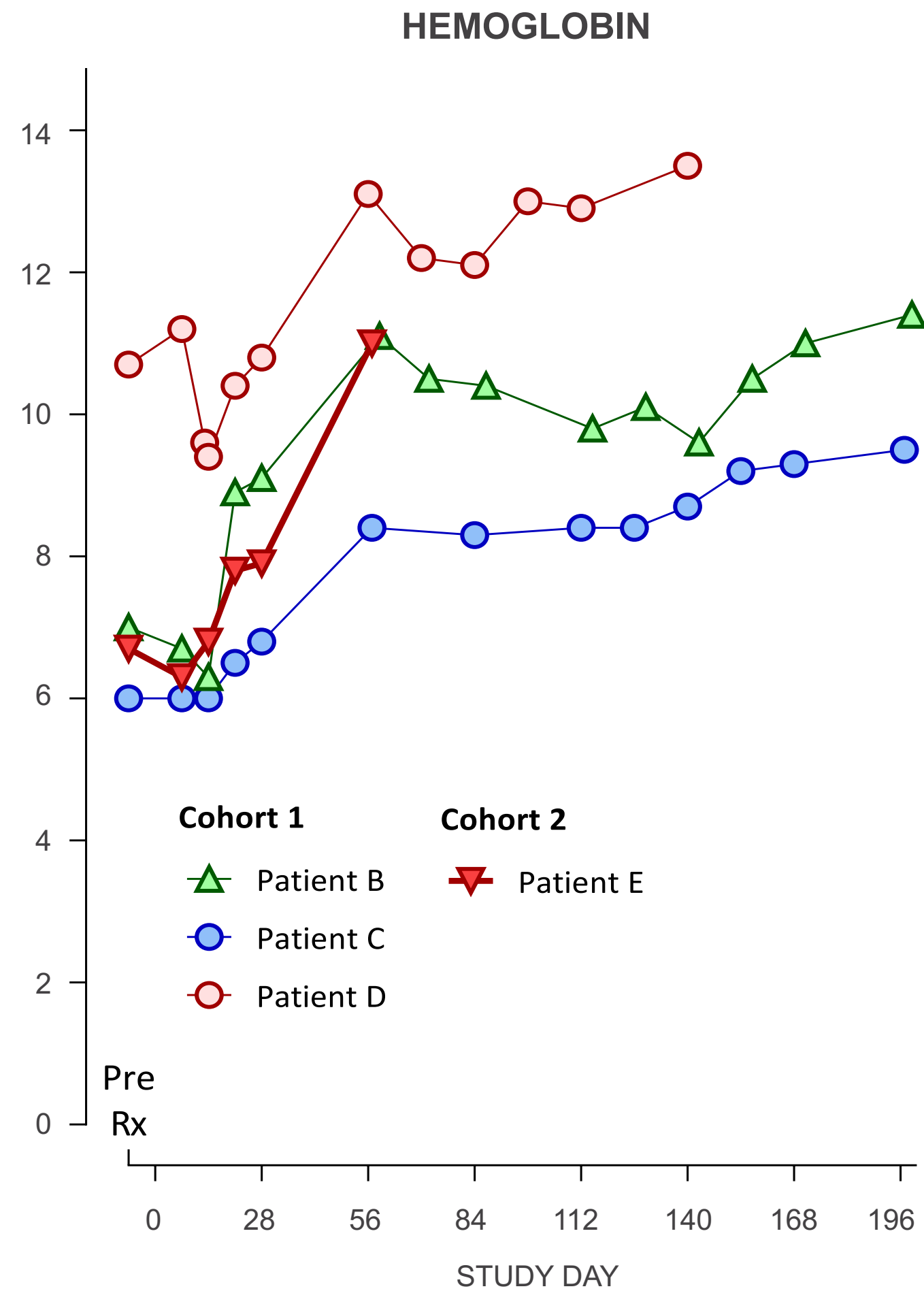
C3 glomerulopathy — understanding a rare complement-driven renal disease

Richard J. H. Smith^{1*}, Gerald B. Appel², Anna M. Blom³, H. Terence Cook⁴, Vivette D D'Agati⁵, Fadi Fakhouri⁶, Véronique Fremeaux-Bacchi⁷, Mihály Józsi⁸, David Kavanagh⁹, John D. Lambris¹⁰, Marina Noris¹¹, Matthew C. Pickering¹², Giuseppe Remuzzi^{11,13,14}, Santiago Rodriguez de Córdoba¹⁵, Sanjeev Sethi¹⁶, Johan Van der Vlag¹⁷, Peter F. Zipfel^{18,19} and Carla M. Nester¹

NATURE REVIEWS | NEPHROLOGY

VOLUME 15 | MARCH 2019 | 129

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).



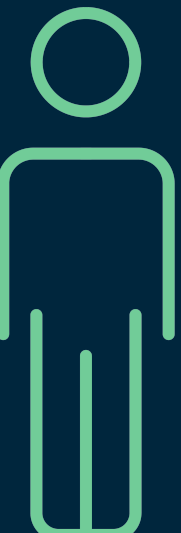
PNH
9930



FOP
9250



ANCA
Vasculitis
9930



Lupus
nephritis
9930



C3G
9930

**A FULL
PIPELINE**



aHUS
9930



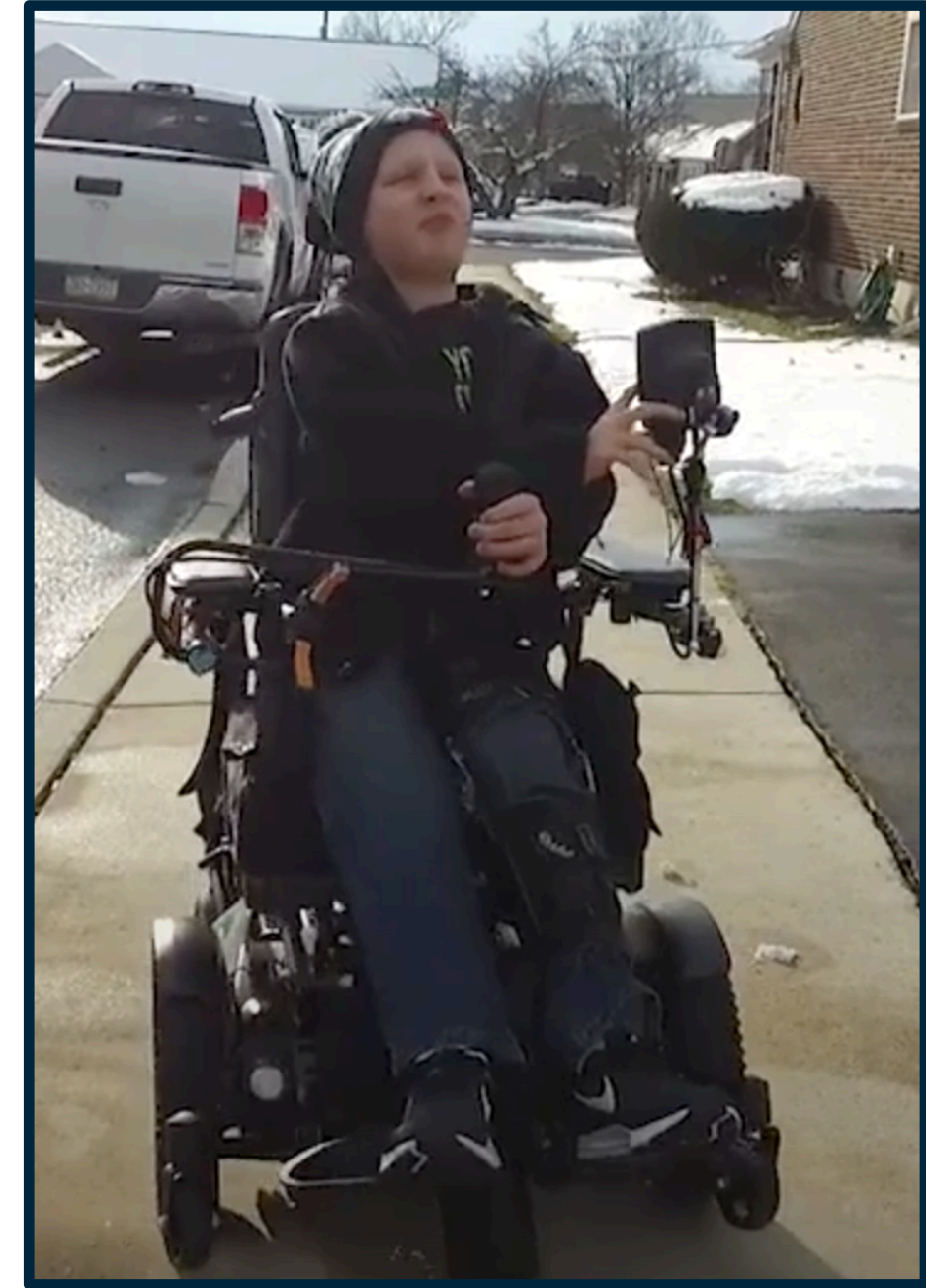
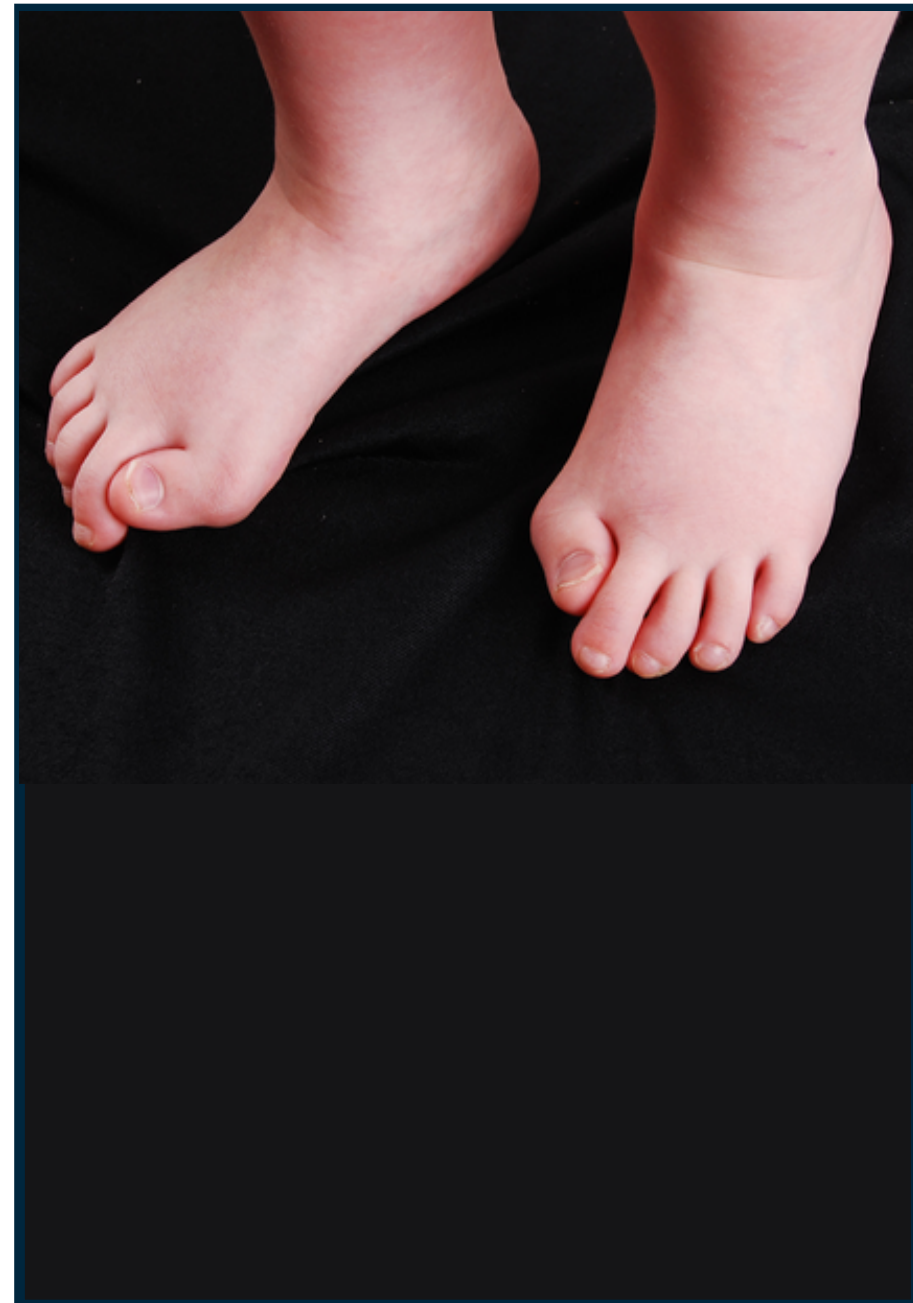
IgAN
9930



PMN
9930

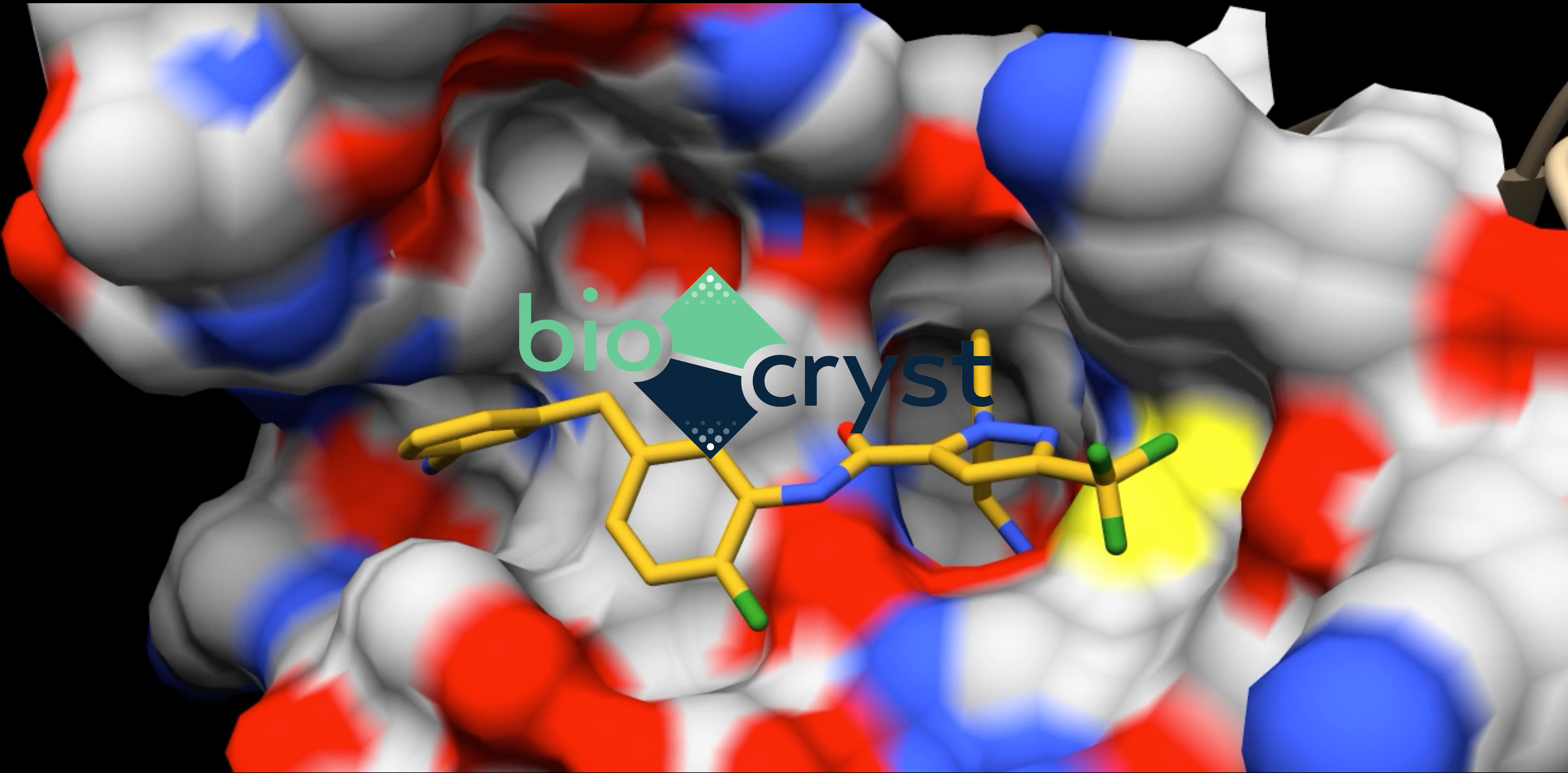


IgAN
vasculitis
9930



ifopa.org





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Significant Upcoming Milestones in 2021

Q1 2021

Approval decision on ORLADEYO in Japan (January 2021)

Data from completed BCX9930 dose ranging study in PNH

Q2 2021

Approval decision on ORLADEYO in EU

Revenues reported from Q1/first full quarter of ORLADEYO sales in US

Launch of ORLADEYO in Japan

Launch of ORLADEYO in Germany

Q3 2021

BCX9930 Advanced Development Trials

BCX9250 Next Steps

ORLADEYO Revenues

This is big.

This is bigger.