

---

---

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

---

**FORM 8-K**

---

**CURRENT REPORT**  
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported) December 23, 2019

---

**CERECOR INC.**

(Exact name of registrant as specified in its charter)

---

Delaware

(State or other jurisdiction of incorporation)

001-37590  
(Commission File Number)

45-0705648  
(IRS Employer Identification No.)

540 Gaither Road, Suite 400, Rockville, Maryland 20850  
(Address of principal executive offices) (Zip Code)

Registrant's Telephone Number, Including Area Code: (410) 522-8707

---

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.001 Par Value	CERC	Nasdaq Capital Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging Growth Company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

---

---

**Item 8.01. Other Events.**

On December 23, 2019, Cerecor Inc. (“Cerecor”) posted an updated Investor Presentation regarding the business of the combined company after completion of the previously announced proposed merger with Aevi Genomic Medicine, Inc. (“Aevi”) to its website at [www.cerecor.com](http://www.cerecor.com). A copy of the investor presentation is attached hereto as Exhibit 99.1.

**Additional Information about the Merger and Where to Find It**

This document does not constitute an offer to sell or the solicitation of an offer to buy any securities of Aevi or Cerecor or the solicitation of any vote or approval. In connection with the proposed Merger, on December 20, 2019, Cerecor filed with the SEC a Registration Statement on Form S-4 containing a proxy statement/prospectus. The proxy statement/prospectus contains important information about Aevi, Cerecor, the Merger and related matters. Aevi will mail or otherwise deliver the proxy statement/prospectus to its stockholders when it becomes available. Investors and security holders of Aevi and Cerecor are urged to read carefully the proxy statement/prospectus relating to the Merger (including any amendments or supplements thereto) in its entirety, because it contains important information about the proposed Merger.

Investors and security holders of Aevi and Cerecor will be able to obtain free copies of the proxy statement/prospectus for the proposed Merger (when it is available) and other documents filed with the SEC by Aevi and Cerecor through the website maintained by the SEC at [www.sec.gov](http://www.sec.gov). In addition, investors and security holders of Aevi will be able to obtain free copies of the proxy statement/prospectus for the proposed Merger (when it is available) by contacting Aevi, Attn: Mike McInaw, [michael.mcinaw@aevigenomics.com](mailto:michael.mcinaw@aevigenomics.com). Investors and security holders of Cerecor will be able to obtain free copies of the proxy statement/prospectus for the merger by contacting Cerecor, Attn: James Harrell, [jharrell@cerecor.com](mailto:jharrell@cerecor.com).

**Participants in the Merger**

Aevi, Cerecor and certain of their respective directors and executive officers may be deemed to be participants in the solicitation of proxies from the stockholders of Aevi in respect of the transactions contemplated by the Merger Agreement between Aevi and Cerecor. Information regarding Aevi’s directors and executive officers is contained in Aevi’s Annual Report on Form 10-K for the fiscal year ended December 31, 2018, which was filed with the SEC on March 29, 2019, and is available in the proxy statement/prospectus that was filed by Cerecor with the SEC in connection with the proposed Merger. Information regarding Cerecor’s directors and executive officers is contained in Cerecor’s Annual Report on Form 10-K for the fiscal year ended December 31, 2018, which was filed with the SEC on March 18, 2019, and in the proxy statement/prospectus that was filed by Cerecor with the SEC in connection with the proposed Merger.

**Cautionary Statement Regarding Forward-Looking Statements**

This document contains forward-looking statements within the meaning of Section 27A of the Securities Act, Section 21E of the Exchange Act and as that term is defined in the Private Securities Litigation Reform Act of 1995, including, but not limited to, Aevi’s and Cerecor’s expectations or predictions of future financial or business performance or conditions. Forward-looking statements are sometimes identified by their use of the terms and phrases such as “estimate,” “project,” “intend,” “forecast,” “anticipate,” “plan,” “planning,” “expect,” “believe,” “will,” “will likely,” “should,” “could,” “would,” “may” or the negative of such terms and other comparable terminology. These forward-looking statements are subject to numerous assumptions, risks and uncertainties, which change over time, are difficult to predict and are generally beyond the control of either company. Actual results may differ materially from current projections.

Important factors that may cause actual results to differ materially from the results discussed in the forward-looking statements or historical experience include risks and uncertainties, including the timing and completion of the Merger, the parties’ ability to satisfy the closing conditions of the Merger Agreement, the failure by Aevi or Cerecor to secure and maintain relationships with collaborators and/or investors; risks relating to clinical trials; risks relating to the commercialization, if any, of Aevi’s or Cerecor’s proposed product candidates (such as

marketing, regulatory, product liability, supply, competition, and other risks); dependence on the efforts of third parties; dependence on intellectual property; and risks that Aevi or Cerecor may lack the financial resources and access to capital to fund proposed operations. Further information on the factors and risks that could affect Aevi's and Cerecor's respective businesses, financial conditions and results of operations are contained in Aevi's and Cerecor's filings with the SEC, which are available at [www.sec.gov](http://www.sec.gov). The forward-looking statements represent Aevi's and Cerecor's estimate as of the date hereof only, and Aevi and Cerecor specifically disclaim any duty or obligation to update forward-looking statements.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits.

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Investor Presentation</a>

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**CERECOR INC.**

Date: December 23, 2019

/s/ Joseph M. Miller

\_\_\_\_\_  
Joseph M. Miller

Chief Financial Officer



# Driven by Science Inspired by Hope

Establishing a leading, rare disease-focused biopharmaceutical company to deliver impactful new medicines to patients

December | 2019



## Forward-Looking Statements

---

This presentation may include forward-looking statements made pursuant to the Private Securities Litigation Reform Act of 1995. Forward-looking statements are statements that are not historical facts. Such forward-looking statements are subject to significant risks and uncertainties that are subject to change based on various factors (many of which are beyond Cerecor, Inc. (“Cerecor”) or Aevi Genomic Medicine, Inc.’s (“Aevi”) control, which could cause actual results to differ from the forward-looking statements. Such statements may include, without limitation, statements with respect to Cerecor’s plans, objectives, projections, expectations and intentions and other statements identified by words such as “projects,” “may,” “will,” “could,” “would,” “should,” “continue,” “seeks,” “aims,” “predicts,” “believes,” “expects,” “anticipates,” “estimates,” “intends,” “plans,” “potential,” or similar expressions (including their use in the negative), or by discussions of future matters such as: Aevi and Cerecor’s respective 2019 outlook; the development of product candidates; timing and success of trial results and regulatory reviews; potential attributes and benefits of product candidates; the expansion of Cerecor’s drug portfolio; the benefits of the proposed merger with Aevi described herein; and other statements that are not historical.

These statements are based upon the current beliefs and expectations but are subject to significant risks and uncertainties, including: drug development costs, timing and other risks; the companies’ cash position and the need to raise additional capital; risks associated with dispositions and acquisitions, such as the pediatric asset sale and the proposed merger with Aevi, including their consumption of company resources to complete, risks they might not close, and the need to quickly and successfully reorganize operations and integrate acquired assets and personnel if they do; reliance on and the need to attract, integrate and retain key personnel; and those other risks detailed in Cerecor’s and Aevi’s respective filings with the Securities and Exchange Commission. Actual results may differ from those set forth in the forward-looking statements. Except as required by applicable law, Cerecor and Aevi expressly disclaim any obligations or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in Cerecor’s or Aevi’s expectations with respect thereto or any change in events, conditions or circumstances on which any statement is based.



# Additional Information Concerning the Merger

---

## Additional Information about the Merger and Where to Find It

In connection with the proposed merger with Aevi, Cerecor will file with the SEC a Registration Statement on Form S-4 containing a proxy statement/prospectus. The proxy statement/prospectus will contain important information about Aevi, Cerecor, the merger and related matters. Aevi will mail or otherwise deliver the proxy statement/prospectus to its stockholders when it becomes available. Investors and security holders of Aevi and Cerecor are urged to read carefully the proxy statement/prospectus relating to the merger (including any amendments or supplements thereto) in its entirety when it is available, because it will contain important information about the proposed merger.

Investors and security holders of Aevi and Cerecor will be able to obtain free copies of the proxy statement/prospectus for the proposed merger (when it is available) and other documents filed with the SEC by Aevi and Cerecor through the website maintained by the SEC at [www.sec.gov](http://www.sec.gov). In addition, investors and security holders of Aevi will be able to obtain free copies of the proxy statement/prospectus for the proposed merger (when it is available) by contacting Aevi, Attn: Mike McInaw, [michael.mcinaw@aevigenomics.com](mailto:michael.mcinaw@aevigenomics.com). Investors and security holders of Cerecor will be able to obtain free copies of the proxy statement/prospectus for the merger by contacting Cerecor, Attn: James Harrell, [jharrell@cerecor.com](mailto:jharrell@cerecor.com).

## Participants in the Merger

Aevi, Cerecor and certain of their respective directors and executive officers may be deemed to be participants in the solicitation of proxies from the stockholders of Aevi in respect of the transactions contemplated by the Merger Agreement between Aevi and Cerecor. Information regarding Aevi's directors and executive officers is contained in Aevi's Annual Report on Form 10-K for the fiscal year ended December 31, 2018, which was filed with the SEC on March 29, 2019, and will also be available in the proxy statement/prospectus that will be filed by Cerecor with the SEC in connection with the proposed merger. Information regarding Cerecor's directors and executive officers is contained in Cerecor's Annual Report on Form 10-K for the fiscal year ended December 31, 2018, which was filed with the SEC on March 18, 2019, and will also be available in the proxy statement/prospectus that will be filed by Cerecor with the SEC in connection with the proposed merger.

## Non-Solicitation

This presentation shall not constitute an offer to sell or the solicitation of an offer to sell or the solicitation of an offer to buy any securities, nor shall there be any sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offer of securities shall be made except by means of a prospectus meeting the requirements of Section 10 of the Securities Act, as amended.

## Cerecor Continues Transformation

---

### Building a rare disease biotech company committed to developing innovative medicines in areas of high unmet need



Advancing CERC-800s to NDA filings and FDA approvals over the next 18 to 24 months; potential PRVs could provide non-dilutive capital for R&D investment



Emphasizing R&D with recent sale of pediatric portfolio, narrowing focus and improving the balance sheet; additional optionality with Millipred®



Strategically positioning the company as a leader in orphan disease drug development to deliver high impact medicines to underserved patient populations



*Identified AEVI Genomic Medicine, Inc. (NASDAQ: GNMX) as a potential target for M&A due to complimentary fit of rare disease pipeline assets and management personnel*



Advancing CNS pipeline to value inflection for strategic optionality as partnered or out-licensed assets





# Cerecor (CERC) & Aevi (GNMX) Have Entered Into a Merger Agreement to Merge

**Propose to form a leading biopharmaceutical company focused on pediatric orphan diseases**

6

Rare disease programs establish robust pipeline with addition of novel, first-in-class medicines

+

4

PRV-eligible, fast-to-market programs with anticipated POC data in 2020, first NDA approval targeted for 2021

+

2

Expansion of leadership team to include CEO Michael Cola and CMO Garry Neil

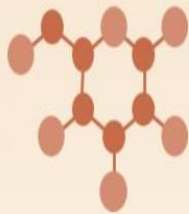
=

1

Leading biopharmaceutical company with *a world-class orphan disease pipeline*

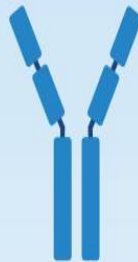


# Combined Company Well-Positioned to Execute on Delivering Innovative New Medicines to Patients



## CERC-800s

- Substrate replacement therapies for CDGs
- 2 pivotal-ready programs with FTD
- All 3 programs received RPDD and ODD
- First NDA filing anticipated in 2021
- Estimated collective TAM of >\$100M



## AEVI-007

- Anti-IL-18 mAb for auto-inflammatory diseases
- Phase 2-ready
- Strong biological rationale evidenced by serum biomarkers
- First NDA filing anticipated in 2022
- Estimated \$400 – 500M PYS in Top 7 WW markets



## AEVI-006

- mTORC1/2 inhibitor for Lymphatic Malformations
- Phase 2-ready
- Existing clinical proof-of-concept with off-label use of sirolimus
- First NDA filing anticipated in 2023
- Estimated \$450 – 900M PYS in US market

Aevi's R&D efforts include working with the Center for Applied Genomics (CAG) at Children's Hospital of Philadelphia (CHOP)



6| CDG: Congenital Disorders of Glycosylation; RPDD: Rare Pediatric Disease Designation; ODD: Orphan Drug Designation  
FTD: Fast Track Designation; TAM: Target Addressable Market; PYS: Peak Year Sales



# Emerging Clinical-Stage, Rare Disease Pipeline

## 6 Programs in Orphan Diseases with Significant Unmet Needs

- Building a rare disease biotech company focused on developing innovative medicines
- Opportunity for multiple novel product launches through 2023

				Development Stage		
Program	Mechanism of Action	Lead Indication	Preclinical	Phase 1	Upcoming Milestone	
	CERC-801*	D-Galactose replacement	PGM1-CDG	Pivotal Study Ready	Initial data from CDG-FIRST 1H20	
	CERC-802*	D-Mannose replacement	MPI-CDG	Pivotal Study Ready		
	CERC-803*	L-Fucose replacement	SLC35C1-CDG	IND-Enabling		
	AEVI-007	Anti-IL-18 mAb	Auto-inflammatory diseases (AOSD, MM)	Phase 1/2 Ready	Initial POC 4Q20/1Q21	
	AEVI-006**	mTORC1/2 inhibitor	Complex Lymphatic Malformations	Phase 1/2 Ready	Initial POC 4Q20/1Q21	
	AEVI-002	Anti-LIGHT mAb	Pediatric Onset Crohn's Disease	Phase 1/2 Study Ongoing	Initial POC 1H20	

\*Rare Pediatric Disease Designation Granted

7 | \*\*Rare Pediatric Disease Designation Eligible





## Recent BD&L Improves Financials, Emphasizes Pipeline

**Sale of commercial assets provides cash and removes debt overhang, while novel asset acquisitions further strengthen pipeline and focus on R&D**

### CERC BD&L Highlights

**Sale of pediatric commercial portfolio to Aytu Bioscience (NASDAQ:AYTU)**

- Overall deal valued in excess of \$43M
- \$17M upfront composed of \$4.5M in cash & \$12.5M of AYTU preferred stock
- Assumption of Cerecor's outstanding payment obligations payable to Deerfield and other liabilities in excess of \$15M
- Estimated annual expense reduction of \$7 to \$9M associated with commercial sales organization transfer to AYTU

### GNMX BD&L Highlights

**In-licensed two Phase 2-ready programs from large pharma**

- \$500K upfront for WW license to AEVI-006, mTORC1/2 inhibitor for Lymphatic Malformations
- Recently exercised option with AstraZeneca for the WW license to AEVI-007; anti-IL-18 mAb for auto-inflammatory diseases
- Strong biological rationale with well-characterized safety & pharmacology
- Existing clinical proof-of-concept leveraging genetics-based and precision medicine

Right-sized balance sheet and added high probability-of-success, mid-stage clinical programs targeting unmet needs in orphan diseases

# Management Team

## Extensive experience in orphan drug development & commercialization

**Michael F. Cola**  
*Chief Executive Officer*

25+ Years Industry Experience

- President of Specialty Pharmaceuticals, Shire plc
- EVP of Global Therapeutic Business Units and Portfolio Management, Shire plc
- President of the Life Sciences Group, Safeguard Scientifics, Inc.
- Senior positions in Product Development and Commercialization, AstraZeneca

**Dr. Pericles Calias**  
*Chief Scientific Officer*

20+ Years Industry Experience

- VP Global CMC & Development, Sucampo Pharmaceuticals
- CSO, Pharming Group
- Senior Director Rare CNS Diseases and Device Lead, Shire plc
- Senior Director Drug Delivery and Chemistry, Eyetech Pharmaceuticals

**Joseph Miller**  
*Chief Financial Officer*

20+ Years Industry Experience

- VP of Finance, Sucampo Pharmaceuticals
- Senior Director of Accounting, Qiagen
- Chief Financial Officer, Eppendorf 5Prime
- Certified Public Accountant

**Dr. Garry A. Neil**  
*Chief Medical Officer*

25+ Years Industry Experience

- Corporate VP of Science & Technology, Johnson & Johnson
- Group President, Johnson & Johnson Pharmaceutical Research and Development
- VP of R&D, Merck KGaA/EMD Pharmaceuticals
- VP of Clinical Research, AstraZeneca







# CERC-800s

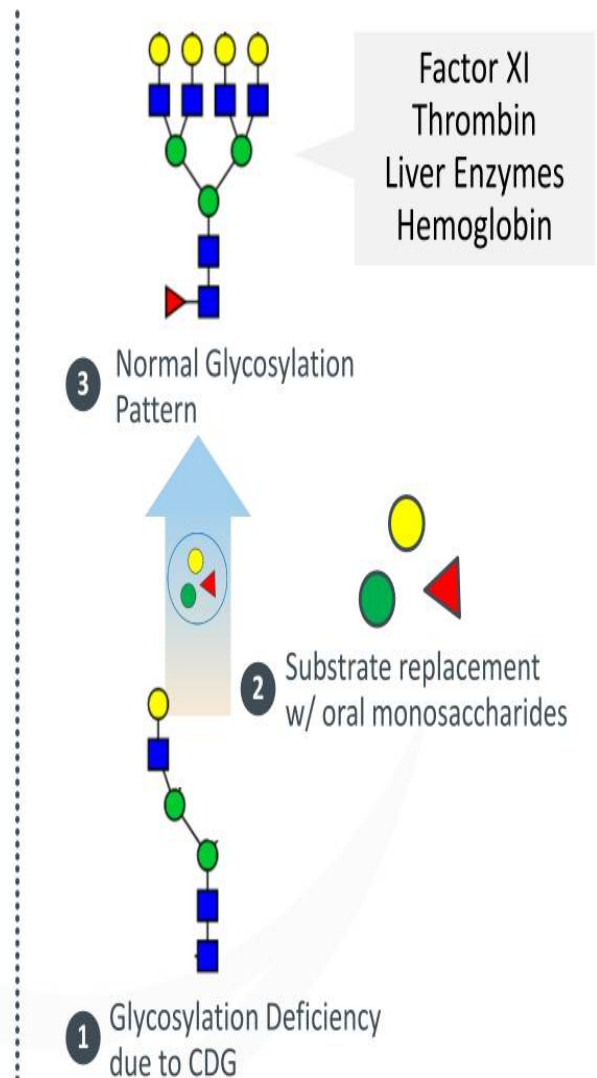
Fast-to-market substrate replacement therapies for  
Congenital Disorders of Glycosylation



# Congenital Disorders of Glycosylation (CDG): Life-Threatening, Ultra-Orphan, Inborn Errors of Metabolism (IEMs)

**Autosomal recessive diseases that result in impaired glycoprotein production and function, but are treatable with substrate replacement therapy**

-  Glycosylation is essential for **protein structure & function**, particularly for circulating proteins and enzymes such as hormones and coagulation factors
-  Due to a mutation in one of more than 100 genes (including *PGM1*, *MPI*, and *SLC35C1*), CDG patients lack the ability to synthesize functioning glycoproteins
-  **Life-threatening multi-system diseases:** developmental delay, hypotonia, neurologic abnormalities, hepatic disease, and coagulopathy
-  **Substrate replacement** via oral monosaccharides results in clinical benefit in many patients

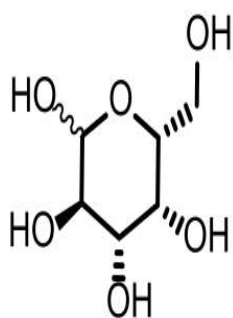


# CERC-800s

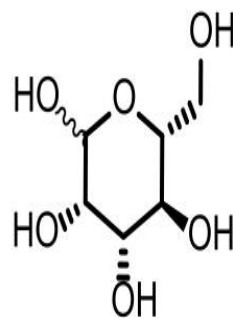
## Substrate replacement therapies for CDGs

### Oral, small molecule, naturally-occurring monosaccharides used as standard-of-care for CDGs

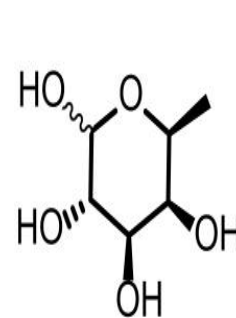
- Rapid onset of action
- Safe and well-tolerated
- Documented clinical experience of efficacy
- Symptoms return upon treatment withdrawal



● D-Galactose



● D-Mannose

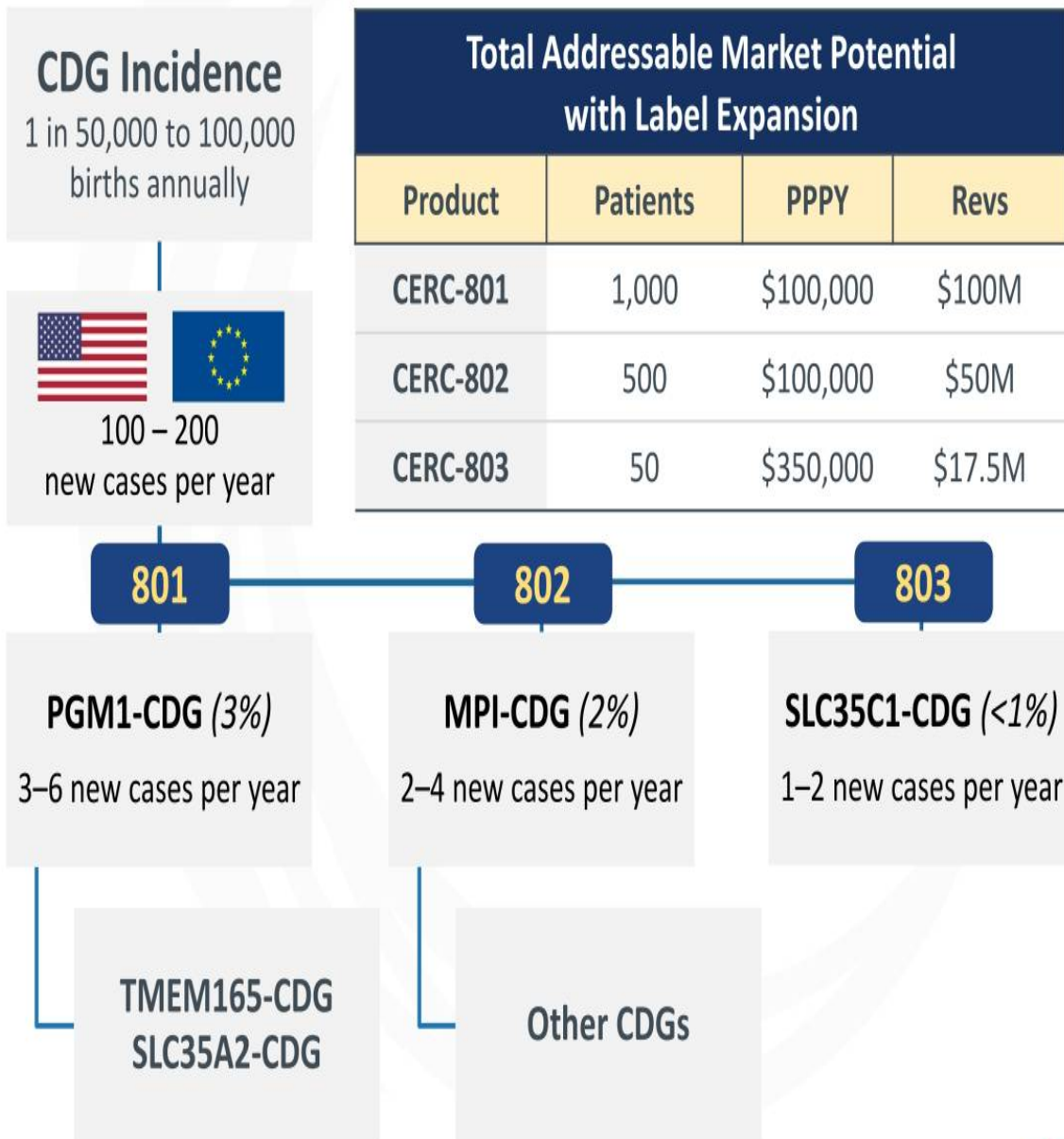


◀ L-Fucose

Eligibility	CERC-801	CERC-802	CERC-803
Accelerated Pathway	✓	✓	✓
NCE 5-yrs Exclusivity	✓	✓	✓
FDA ODD 7-yrs Exclusivity	✓	✓	✓
EMA ODD 10-yrs Exclusivity	✓	✓	✓
Priority Review Voucher	✓	✓	✓

# CDG Addressable Markets & Commercial Opportunity

Opportunity for indication expansion to build a >\$100mm franchise



13| Peanne et al. *Eur J Med Genet.* 2017, Kjaergaard. *Dan Med Bull.* 2004, 51, pp. 350-63





## CDG FIRST Trial: Retrospective Study to Accelerate Development & Approval of CERC-800s; Targeted NDA Filing(s) Starting in 2021

**Multi-center, international, non-interventional, retrospective study of CDG patients who have been treated with unapproved sugar supplements**



Retrospective Chart Reviews & Registry Data Have Been Successfully Used to Minimize or Obviate Prospective Clinical Studies

Data to be collected: *Natural History, Safety & Treatment Outcomes*



CDG Connect Patient Insights Network (PIN)  
<https://connect.invitae.com/org/cdg>



## Key Upcoming CERC-800s Milestones

### Multiple value-generating inflection points over the next 12 to 18 months

- FDA meetings to define pathway to NDA submission and approval
  - Briefing package expected to include data collected under CDG FIRST
  - Determine pivotal study requirement and/or design

	Program	Target Indication	Upcoming Milestone
Accelerated NDA Pathway	<b>CERC-801</b> (D-Galactose)	PGM1-CDG	<ul style="list-style-type: none"> <li>• Initial data from CDG FIRST 1H 2020</li> <li>• BTD Request 2Q/3Q 2020</li> <li>• NDA Submission 1H 2021</li> </ul>
	<b>CERC-802</b> (D-Mannose)	MPI-CDG	<ul style="list-style-type: none"> <li>• Initial data from CDG FIRST 1H 2020</li> <li>• BTD Request 2Q/3Q 2020</li> <li>• NDA Submission 2H 2021</li> </ul>
	<b>CERC-803</b> (L-Fucose)	SLC35C1-CDG	<ul style="list-style-type: none"> <li>• Initial data from CDG FIRST 1H 2020</li> <li>• IND Filing 1H 2020</li> <li>• BTD Request 2Q/3Q 2020</li> <li>• NDA Submission 1H 2022</li> </ul>

# AEVI-007

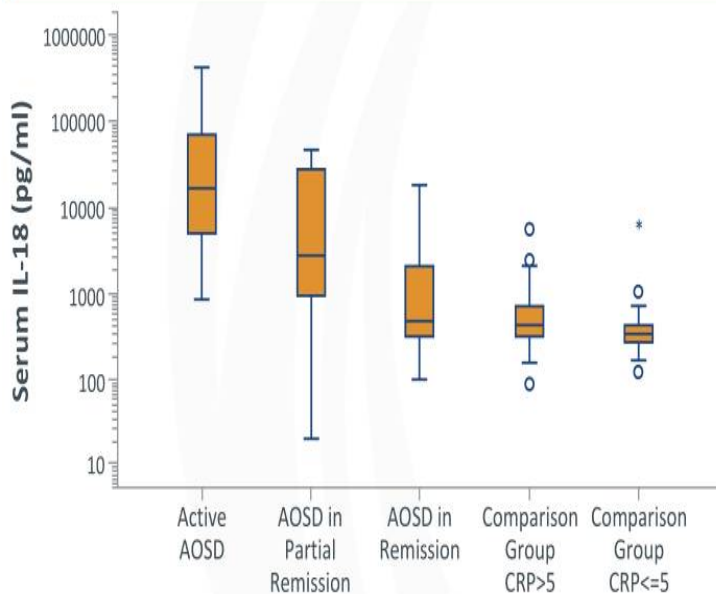
Phase 2-ready, anti-IL-18 monoclonal antibody for  
orphan auto-inflammatory diseases



# IL-18-Mediated Autoimmune Disorders

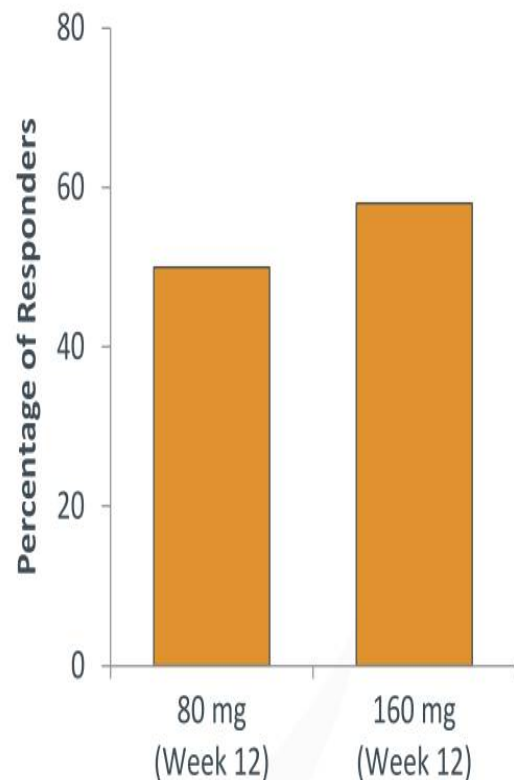
**IL-18 is a pro-inflammatory cytokine produced by macrophages, stimulating production/release of IFN $\gamma$  and regulating many immune processes**

## Elevated Serum IL-18 Levels in AOSD Patients



- **IL-18 is a key driver of several orphan auto-inflammatory diseases**
  - Adult Onset Still’s Disease (AOSD)
  - Multiple Myeloma (MM)
- **Serum IL-18 correlates with disease severity**
  - AB2 Bio clinical proof-of-concept in AOSD (n = 23) using IL-18bp ( $T_{1/2}$  = 40 h); 4/4 patients with undetectable serum IL-18 had a clinical response

## IL-18bp Response Rates



Patients received subcutaneous administration of 80 or 160 mg three times per week

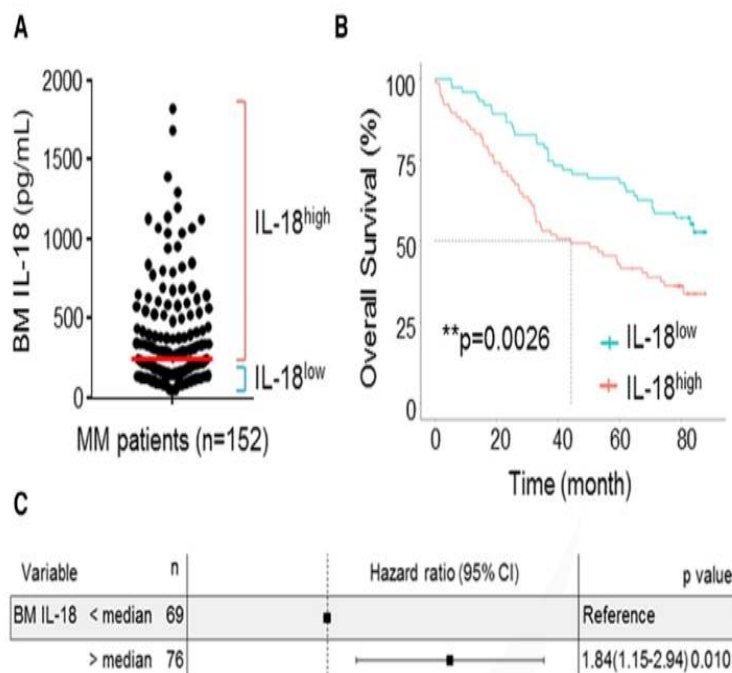


# Elevated IL-18 is Associated with Poor Prognosis in Myeloma

## IL-18 blockade has the potential to significantly prolong survival in multiple myeloma patients with elevated IL-18 levels

- Patients with low IL-18 have significantly longer median survival (>84 mos vs. 42 mos) than patients with high IL-18 ( $p = 0.0026$ , HR = 1.84)
- No association between bone marrow IL-18 levels and classical myeloma risk factors; IL-18 is an *independent determinant* of poor survival
- Reducing IL-18 levels prolongs survival in rodent models
- IL-18 could be a useful biomarker to select patients, determine optimal dose

IL-18 levels are elevated in many MM patients and correlate with poor survival

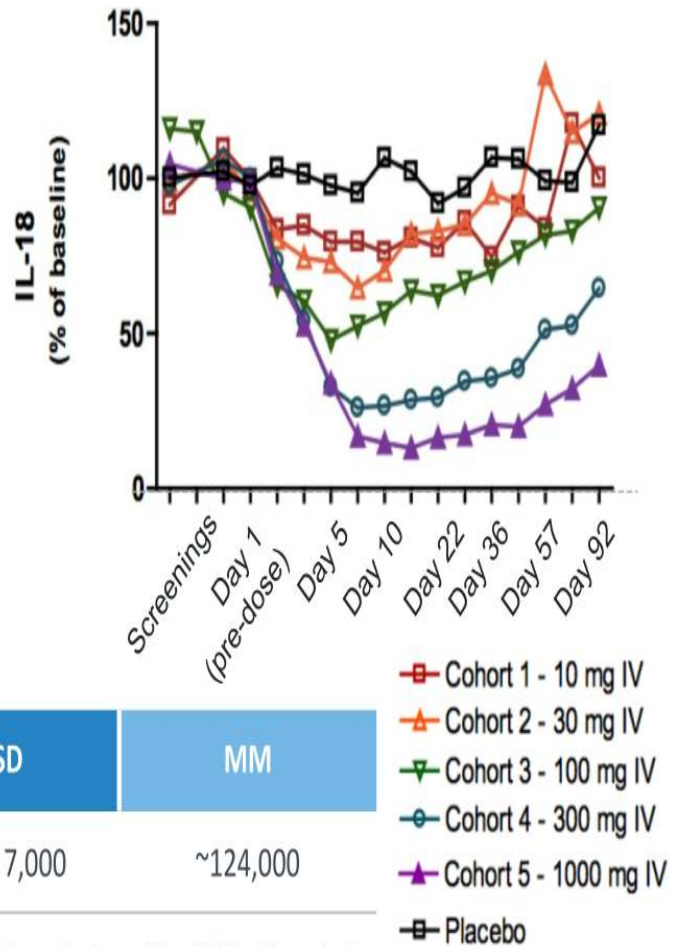


(A) Bone marrow IL-18 levels, (B) Kaplan-Meier survival curve of IL-18 high and IL-18 low patients, (C) Hazard ratio of survival based on bone marrow IL-18 levels

# AEVI-007: Anti-IL-18 mAb Developed by Medimmune

## A high affinity, neutralizing monoclonal antibody against IL-18

- **Potent and durable IL-18 inhibition**
  - Evaluated in Phase 1 SAD for COPD
  - IV doses of 10, 30, 100, 300 or 1000 mg
  - Safe and well-tolerated (n = 31)
- **Phase 2-ready asset**
  - 13-week monkey tox completed
  - Frozen, unformulated bulk material available to support clinical proof-of-concept in patients and nonclinical 6-month chronic tox studies



	AOSD	MM
US prevalence	3,500 – 7,000	~124,000
Estimated % of patients treated	20 – 30% of market	10 – 12% of market
PPPY	\$250,000 – 300,000	\$100,000 – 125,000
Estimated PYS	\$400 – 500M	\$1 – 1.2B*

19 | \*US market only

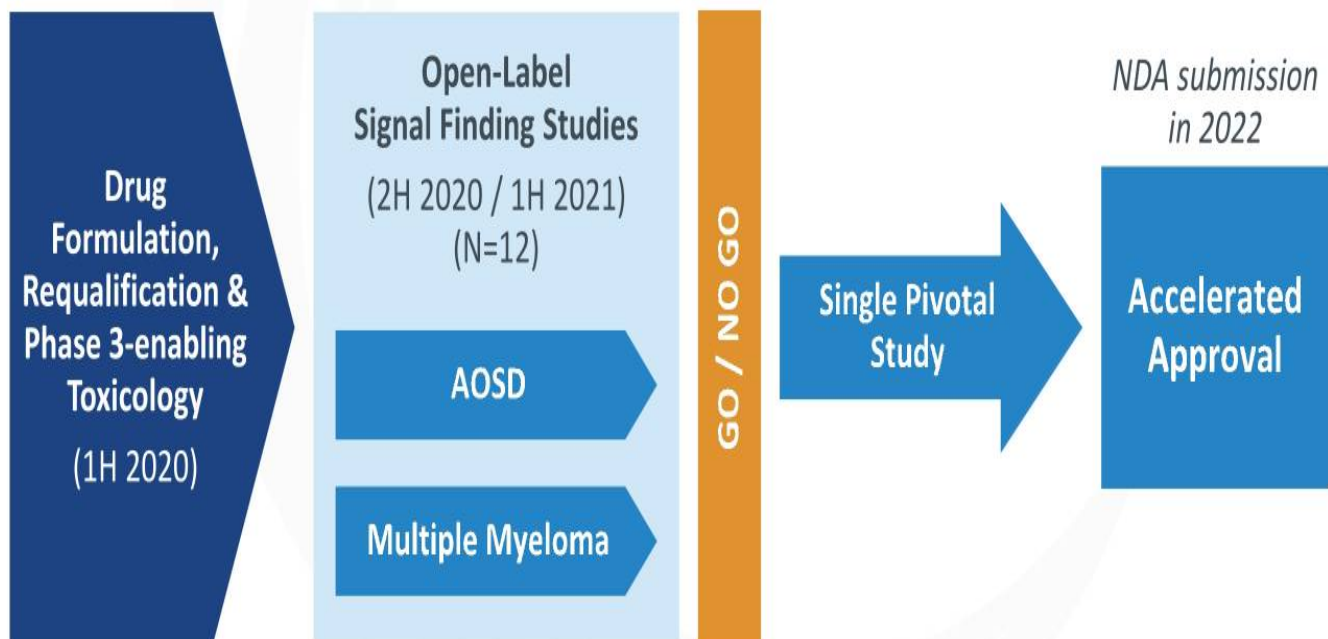




## AEVI-007 Development Plan

### Prioritize lower-risk, less capital intensive development strategy

- Both programs are de-risked by preclinical and clinical proof-of-concept
- Precision medicine approach using disease biomarkers is highly efficient and further de-risks clinical programs
- Opportunity to efficiently demonstrate POC in target patient population(s)



# AEVI-006

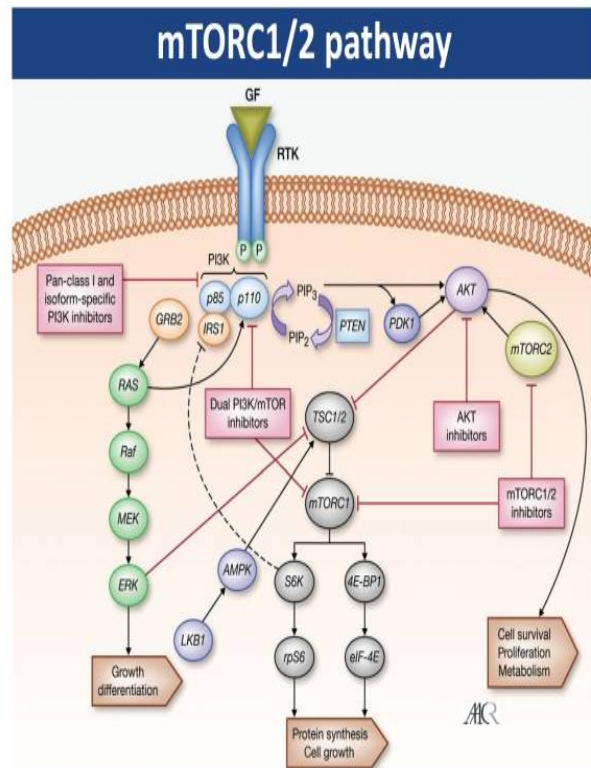
Phase 2-ready, mTORC1/2 small molecule inhibitor  
for complex Lymphatic Malformations



# mTOR Pathway in Lymphatic Malformations (LM)

**Lymphatic Malformations are a family of potentially life-threatening congenital diseases of the lymphatic system**

- **Orphan disease(s) with combined US prevalence of 30,000 to 60,000 patients**
  - Kaposiform Lymphangiomatosis (KLA)
  - Generalized Lymphatic Anomaly (GLA)
  - Gorham Stout Disease (GSD)
  - Conducting Channel Anomalies (CCA)
- **High morbidity and mortality, pediatric diseases with unmet needs**
  - GLA 7-year mortality rate = ~20%
  - KLA median OS = 2.75 years, 5-year OS = ~50%
  - No approved therapy
- **Caused by mutations in PI3K/AKT/mTOR**
  - Clinically meaningful responses with off-label use of mTORC1 inhibitor sirolimus



© 2014 American Association for Cancer Research Molecular Cancer Therapeutics Reviews



Cell Proliferation

Cell Survival

Angiogenesis



1. Estimated from Perkins et al. (2010) *Otolaryngol Head Neck Surg.* 142(6):789-94.
2. Brouillard et al. (2014) *J Clin Invest.* 124(3):898-904.
3. Ozeki et al. (2016) *Pediatr Blood Cancer.* 63(5):832-8.
4. Croteau et al. (2014) *J Pediatr.* 164(2):383-8.
5. Adams et al. (2016) *Pediatrics.* 137(2):e20153257.



## Off-label Use of mTOR Inhibitor Sirolimus in LM

### Open-label clinical studies support efficacy, however use is limited by tolerability issues and lack of FDA approval

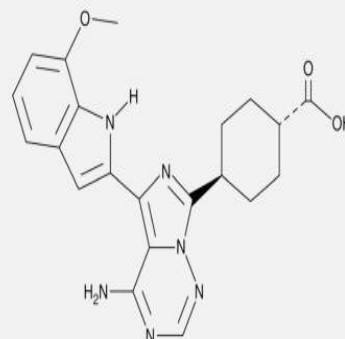
- **Phase 2 trial enrolled patients with complicated vascular anomalies**
  - Study enrolled patients with different subtypes of LM not controlled by previous medication, sclerotherapy and/or surgery
  - Sirolimus was administered orally for 12 courses of 28 days each
  - 57 patients were evaluable for efficacy at the end of course 6, and 53 were evaluable at the end of course 12
- **Safety and tolerability profile leads to low compliance, requires frequent monitoring**
  - Physicians reported that sirolimus causes high rates of stomatitis (~60%)
  - Sirolimus bears black box warning for immunosuppression and malignancies

Overall Response	6-month (n=57)	12-month (n=53)	Grade 2 or > AEs
Complete Response	0	0	<ul style="list-style-type: none"><li>• Blood/bone marrow (50%)</li><li>• Gastrointestinal (55%)</li><li>• Metabolic/laboratory (20%)</li><li>• Infection (15%)</li></ul>
Partial Response	47 (83%)	45 (85%)	
Progressive Disease	7 (12%)	8 (15%)	
Stable Disease	3 (5%)	0	

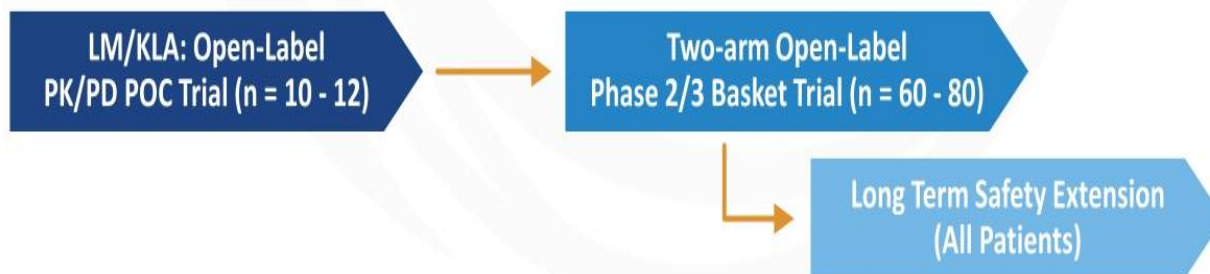
# AEVI-006: mTORC1/2 Inhibitor Developed by Astellas

## High potency, 2<sup>nd</sup> generation, dual inhibitor of mTORC1/2 with potential for improved efficacy and tolerability

- **Phase 2-ready asset**
  - 4-week nonclinical tox studies completed
  - Previously studied in Phase 1 MAD (n = 128)
  - Development discontinued upon determination that target efficacious doses were above MTD (30mg QD)<sup>1</sup>
  - Significantly lower doses than MTD likely required to treat Lymphatic Malformations
- **LM presents an attractive market opportunity**
  - TAM estimated to be 10% of LM patients (~3,000)
  - Estimated PYS for US market = \$450 – 900M
  - PRV eligible, pricing similar to Afinitor (\$190K/yr)



- Dual mTOR inhibitor maximizes impact of mTOR blockade, as mTORC2 is insensitive to rapalogs
- Orally available, ATP-competitive kinase inhibitor; IC<sub>50</sub> = 22 nM and 65 nM for mTORC1 and mTORC2, respectively<sup>2</sup>
- Active against a broad panel of tumor cell lines, with IC<sub>50</sub> values <10 μM, including models resistant to rapalogs<sup>2</sup>



1. Mateo et al. (2016) *Br J Cancer*. 114(8):889-96.  
24 | 2. Bhagwat et al. (2011) *Mol Cancer Ther*. 10(8):1394-406.



**AEVI-002**

Anti-LIGHT monoclonal antibody in clinical studies for  
Severe Pediatric-Onset IBD

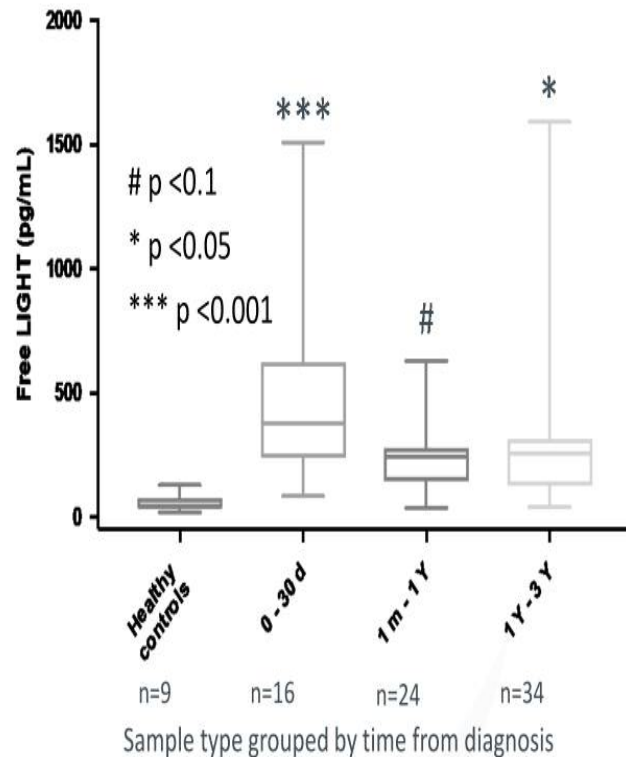


# Elevated LIGHT is Associated with Active Inflammatory Disease

## A novel target for autoimmune disease identified via CHOP discovery

- **LIGHT** (Lymphotoxin-like, exhibits Inducible expression, and competes with HSV Glycoprotein D for HVEM, a receptor expressed by T lymphocytes)
  - Novel TNF superfamily member implicated as a key mediator of inflammation
  - Co-stimulator of T cells and can stimulate a Th1 profile of cytokines, including IFN $\gamma$ <sup>1</sup>
  - Expressed on activated T cells, NK cells, monocytes, granulocytes, and immature dendritic cells<sup>2</sup>
- Elevated free **LIGHT** levels detected in Pediatric Crohn's Disease patients using Aevi's proprietary assay
  - Free LIGHT correlates with disease activity in patient plasma samples from CHOP BioBank
  - Plasma and tissue LIGHT levels are elevated in patients with IBD

### Free LIGHT Levels in Pediatric Crohn's Disease



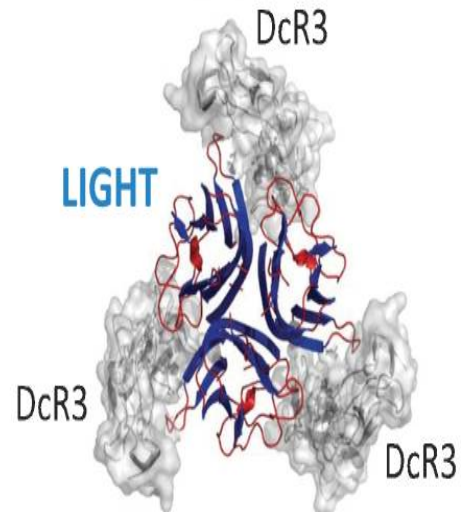
In a non-interventional study of 81 patients undergoing colonoscopy and biopsy, elevated mRNA levels of LIGHT were detected in the inflamed tissues of patients with IBD, compared to healthy individuals

1. Ware CF. (2005) Annu Rev Immunol. 23:787-819.  
26 | 2. Wang & Fu. (2004) Immunol Res. 30(2):201-214.

## AEVI-002: Anti-LIGHT mAb Partnered with KHK

### Clinical-stage, first-in-class monoclonal antibody against LIGHT, originally discovered by La Jolla Allergy Institute

- Fully-human, IgG4 antibody with successfully completed Phase 1 SAD study in healthy volunteers
  - Demonstrated good safety up to 1200 mg single doses
  - Favorable PK profile supportive of q14d, sc dosing
  - 8-week monkey tox showed minimal immunogenicity and benign safety profile
- Anti-LIGHT blockade presents an opportunity for an immuno-regulatory agent with a novel mode of action
  - Initial orphan indication: **Severe Pediatric-Onset IBD**, a life-altering, serious disease with significant unmet need
- Free LIGHT assay developed in collaboration with MyriadRBM enables a precision medicine development approach



- LIGHT homo-trimer binds three DcR3 molecules capable of neutralizing activity
- Anti-LIGHT mAb AEVI-002 binds to the same sites as DcR3, resulting in blockade of signal transduction

Phase 1/2 open-label signal finding study currently ongoing;  
initial data expected 1H 2020

1. Ware CF. (2005) Annu Rev Immunol. 23:787-819.  
27 | 2. Wang & Fu. (2004) Immunol Res. 30(2):201-214.

# Key Financial Information





## Financial & Investor Information

---

### Key financial highlights

#### NASDAQ:CERC

- Share Price (as of 12/16/19) = \$4.47
  - 52-week high = \$7.66
  - 52-week low = \$2.71
- O/S = 45.8M
- Market Cap (as of 12/16/19): \$205M
- Average Daily Volume: 69K
- Pro-Forma Cash as of September 30, 2019 (following the closing of the Aytu transaction) = \$9.1M

#### NASDAQ:GNMX

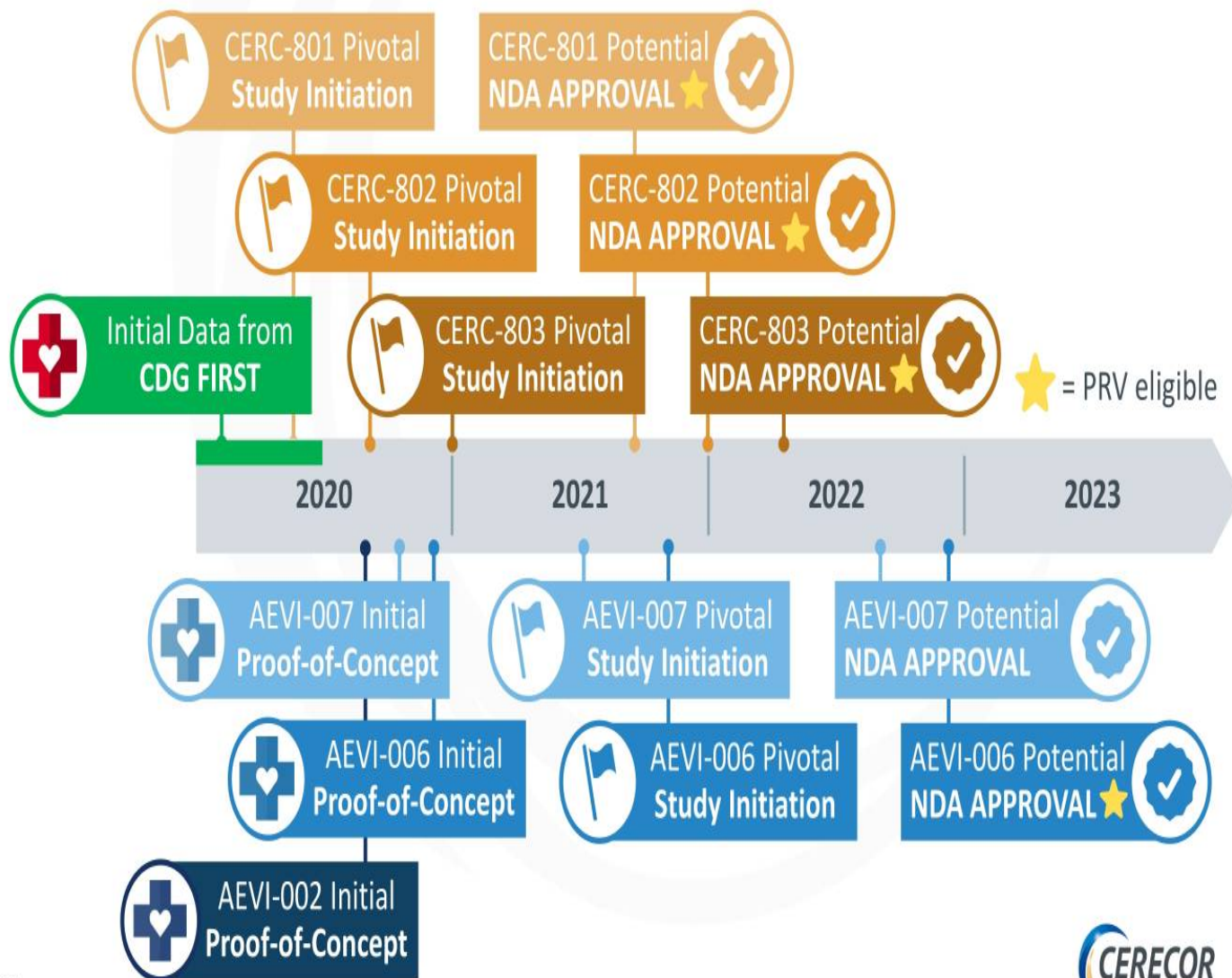
- Share Price (as of 12/16/19) = \$0.14
  - 52-week high = \$1.31
  - 52-week low = \$0.11
- O/S = 64.8M
- Market Cap (as of 12/16/19): \$9M
- Average Daily Volume: 359K

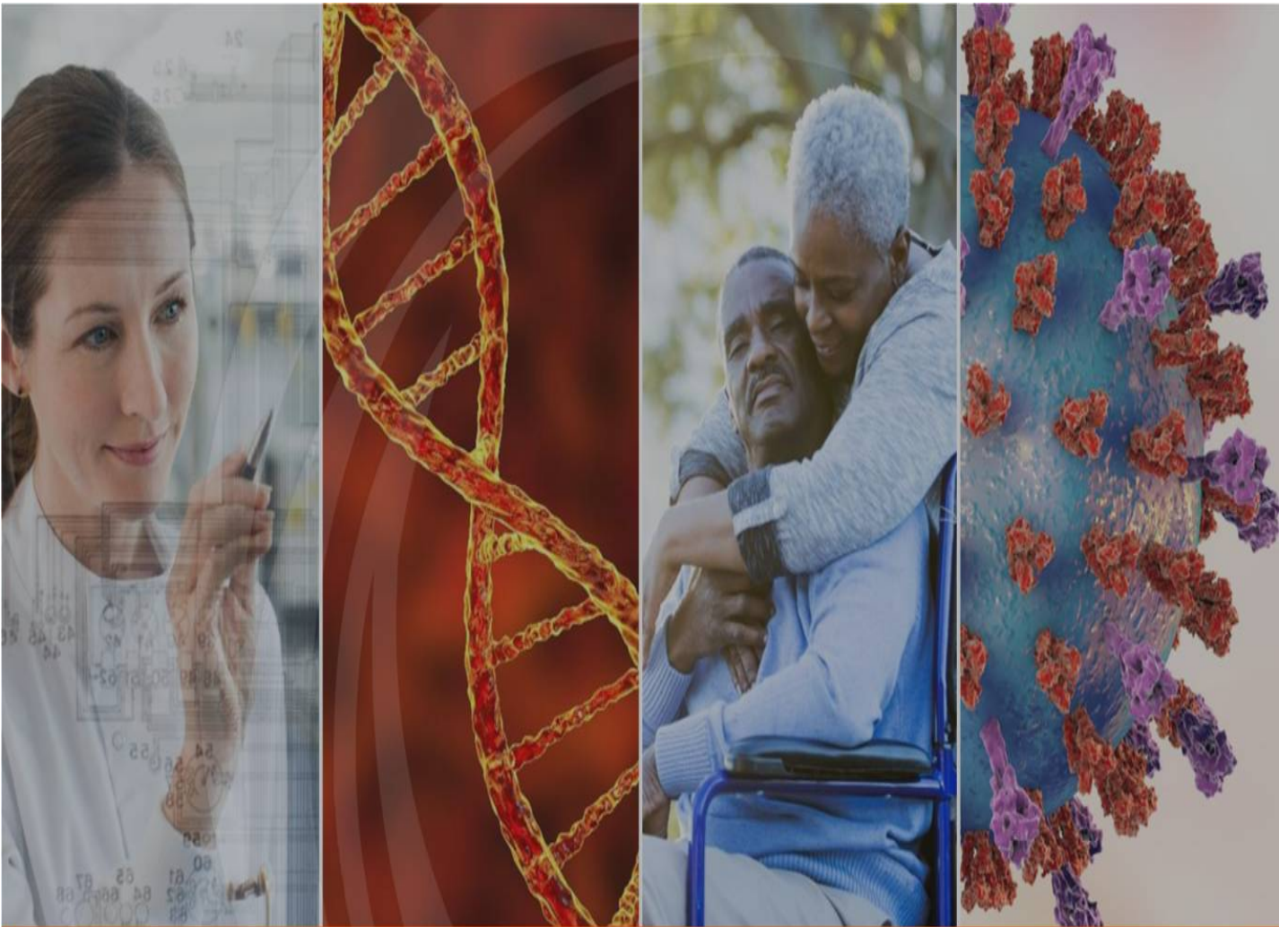
Continued optionality and cash flows from Millipred<sup>®</sup>,  
with a means to assist in funding portfolio assets

# Executive Summary

## Unique potential for multiple drug approvals through 2023

- Execution on orphan pipeline to deliver value for patients and shareholders
  - Attractive commercial opportunities and 4 potential PRV awards from first-in-class medicines for diseases with no approved treatment options





# Driven by Science Inspired by Hope

NASDAQ:CERC

[www.cerecor.com](http://www.cerecor.com)





# Appendix



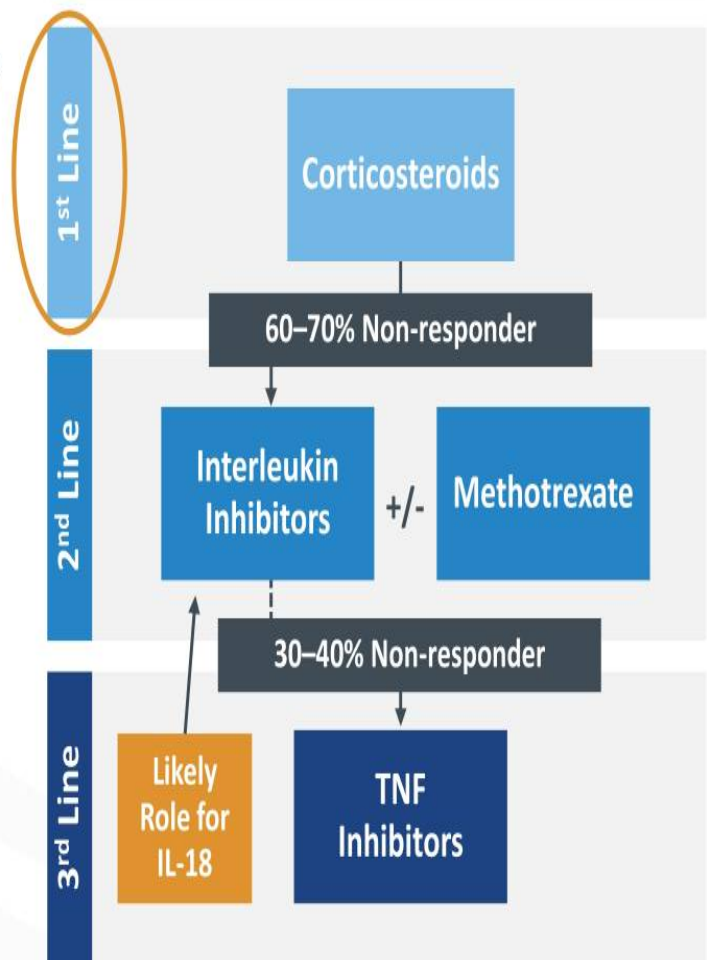


# AOSD Treatment Paradigm

Currently there are no approved targeted therapies for AOSD in the US, and no interventional clinical trials underway

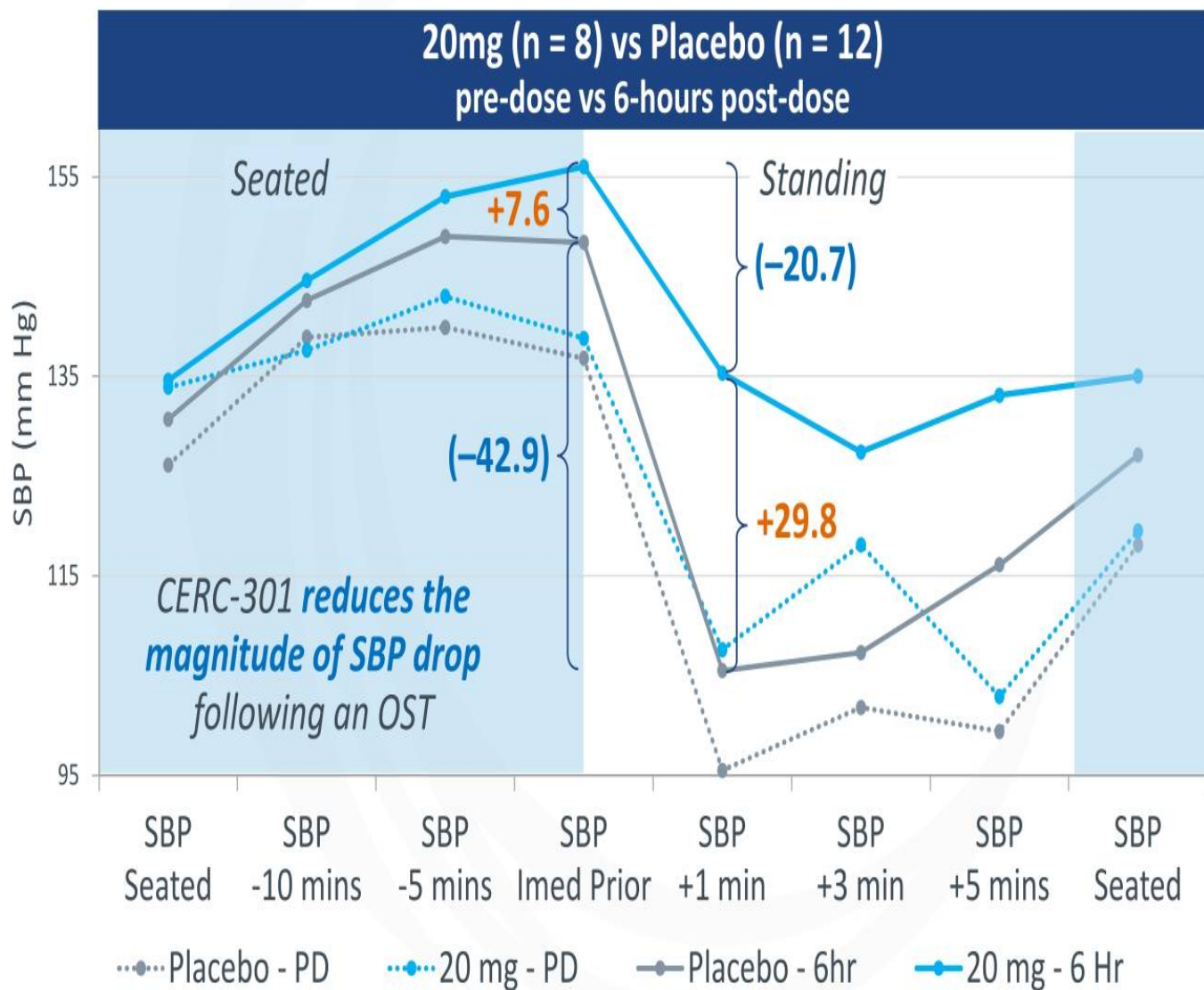
- **Fever, rash, pharyngitis, arthritis, liver disease and increased ferritin**
  - No definitive genetic or infectious cause
  - At least half have severe, chronic disease
  - Elevated serum IL-18<sup>1</sup>
- **Initially treated corticosteroids & NSAIDs; 30 – 40% response rate**
  - Non-responders treated with biologics (anti-IL-1/IL-6) +/- MTX; 30 – 40% still fail to respond
  - Initially position AEVI-007 for use in patients refractory to existing biologics (~ 900 patients in US)
  - Precision medicine approach with predictive biomarkers will facilitate positioning as first-line therapy (2000–2500 patients in the US)

*Opportunity to target sub-population presenting with significantly elevated serum IL-18 for first-line AEVI-007 therapy*



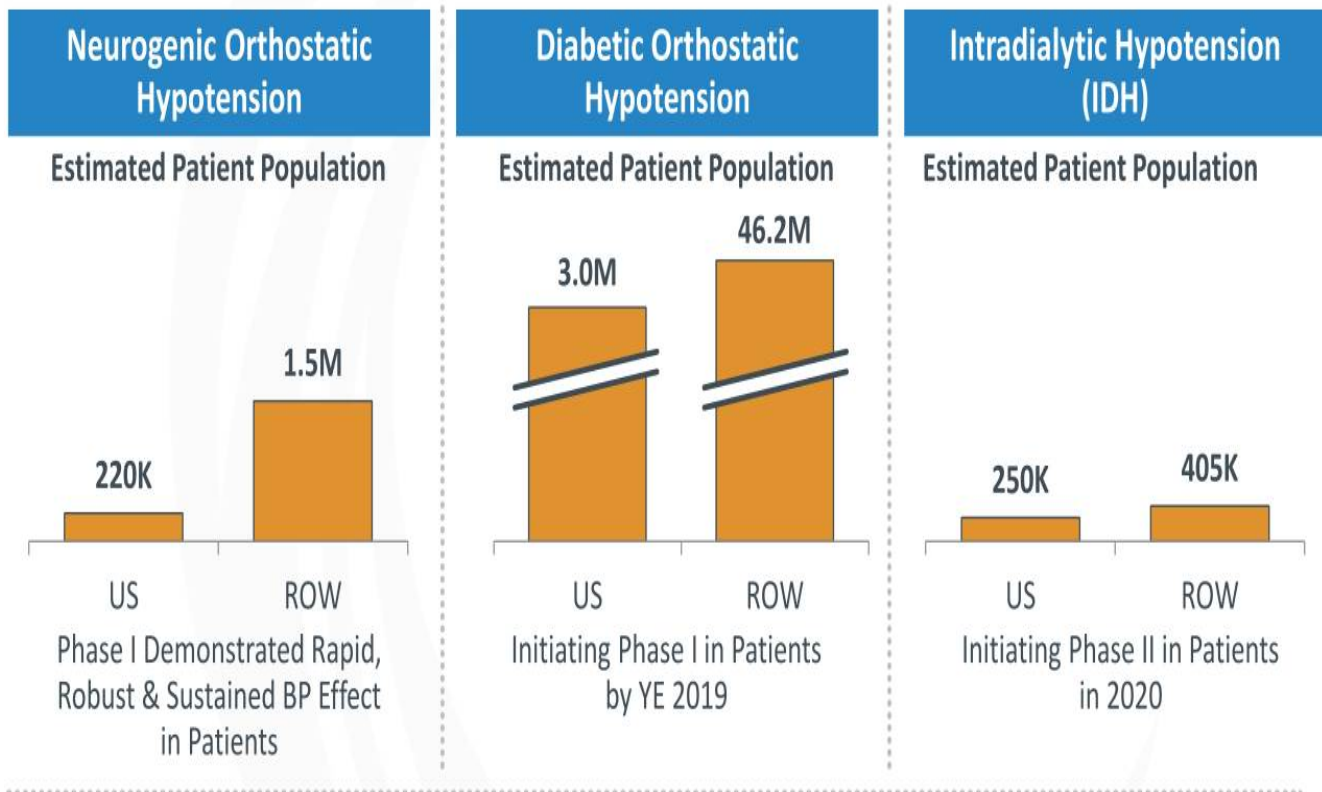
## CERC-301: Phase I Results in nOH Highlighting 20mg Dose

20mg dose results in +29.8mm Hg increase in SBP over placebo at 6-hour post-dose OST, while only increasing +7.6mm Hg while seated



## CERC-301: A Pipeline Within a Product

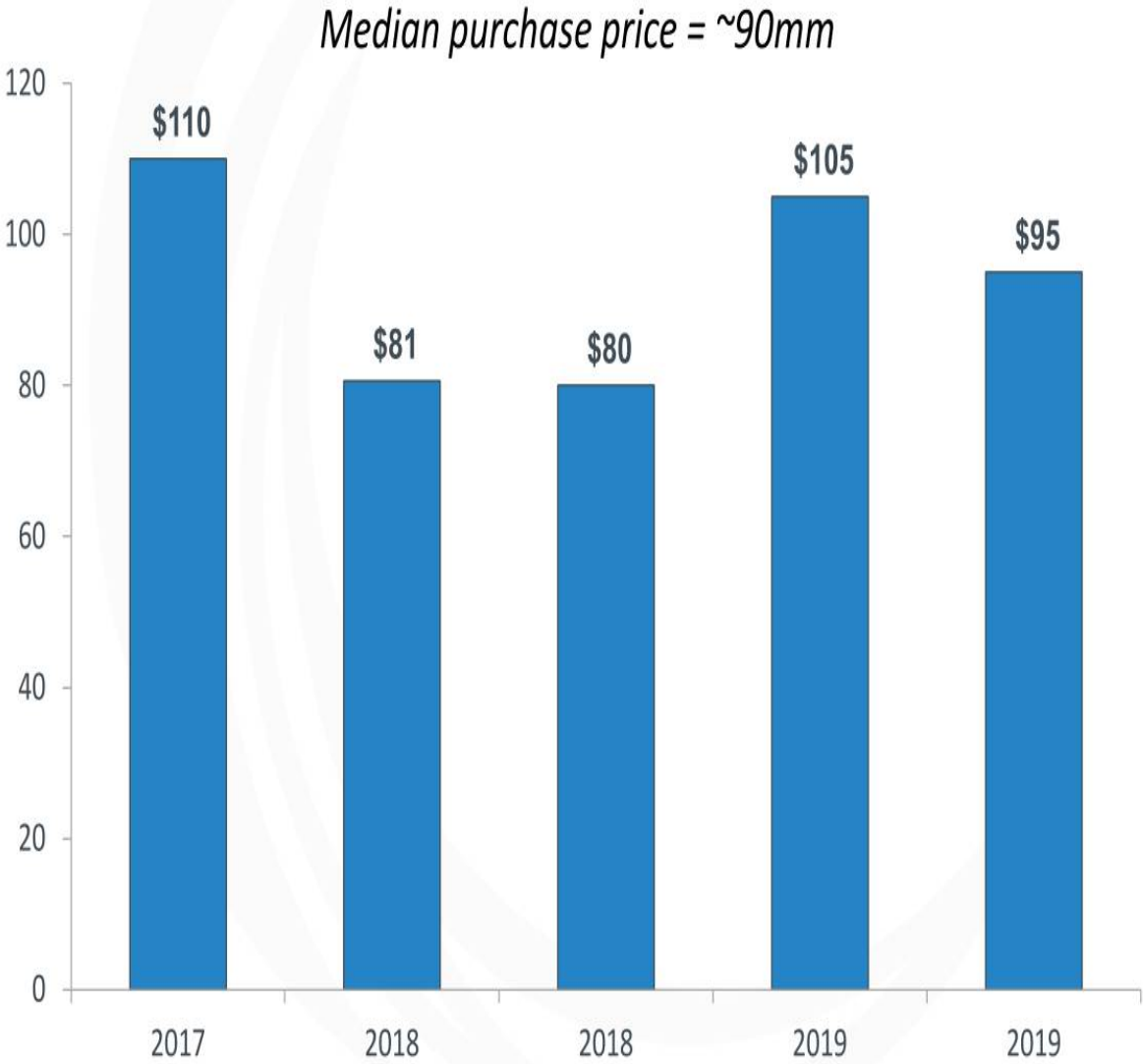
**CERC-301's differentiated profile expands addressable patient population to additional diseases characterized by low blood pressure**



- Large target addressable markets with limited therapeutic options
  - Northera® and midodrine act as direct adrenergic agonists with narrow clinical utility, resulting in unmet medical needs
  - Clinical proof-of-concept throughout 2019 and 2020 will create an opportunity for strategic partnership(s)
  - Seeking strategic partnership to support continued clinical development

# Value of a Priority Review Voucher

## Recent PRV sales demonstrate stabilization in asset valuation



36 | Reference: <https://priorityreviewvoucher.org/>





## Preclinical Pipeline

### Additional early-stage, preclinical programs leveraging novel approaches to diseases with high unmet needs

- Potential for **3** new IND filings throughout 2020

	Program	Mechanism of Action	Lead Indication	Development Stage		
				Discovery	Lead Opt	IND-Enabling
CERC	CERC-406	CNS-Targeted COMT inhibitor	Parkinson's Disease	▶		
	CERC-913	Nucleoside replacement	DGUOK Deficiency	▶		
GIMX	AEVI-005	First-in-class mAb (undisclosed target)	Rare auto-inflammatory	▶		

