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Developing Therapeutics for the Treatment of Serious Rare and Life-Threatening Diseases with Critical Unmet Medical Needs

Corporate Presentation

June 2019

Nasdaq: ACER

Forward-looking Statements

This presentation contains "forward-looking statements" that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements, other than statements of historical facts, included in this presentation regarding strategy, future operations, future financial position, future revenues, projected expenses, regulatory approvals, cash position, liquidity, prospects, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to expectations regarding our capitalization and financial resources; the potential for EDSIVO™ (celiprolol), ACER-001 and osanetant to safely and effectively treat diseases and to be approved for marketing; the commercial or market opportunity in any target indication; the adequacy of our capital to support our future operations and our ability to successfully initiate and complete clinical trials and regulatory submissions; the ability to protect our intellectual property rights; the nature, strategy and focus of our business plans; future economic conditions or performance; and the development, expected timeline, approval and commercial potential of any of our product candidates. We may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements and you should not place undue reliance on these forward-looking statements. Such statements are based on management's current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation, risks and uncertainties associated with the ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the availability of sufficient resources to meet our business objectives and operational requirements, the fact that the results of earlier studies and trials may not be predictive of future clinical trial results, the protection and market exclusivity provided by our intellectual property, risks related to the drug development and the regulatory approval process and the impact of competitive products and technological changes. We disclaim any intent or obligation to update these forward-looking statements to reflect events or circumstances that exist after the date on which they were made. You should review additional disclosures we make in our filings with the Securities and Exchange Commission, including our Quarterly Reports on Form 10-Q and our Annual Report on Form 10-K. You may access these documents for no charge at http://www.sec.gov.

Corporate Overview

Acer Therapeutics is a pharmaceutical company that acquires, develops and seeks to commercialize therapies for serious rare and life-threatening diseases with critical unmet medical needs

- Headquartered: **Newton, MA**
- Headcount: **48**
- Founded: **December 2013**
- Public: **September 2017**
- Cash:
  - **$31.8M** as of March 31, 2019
  - Expected to have sufficient capital into H1 2020

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Executive Leadership Team

Chris Schelling **CEO & Founder**	• 20 years; strategic commercial development & orphan	BIOMARIN
Will Andrews, MD **Chief Medical Officer**	• 20 years; clinical development, medical affairs & orphan • M.D. Yale University School of Medicine	sunovion
Matt Seibt **Interim Head of Commercial**	• 22 years; sales, market access & product launch	Biogen
Harry Palmin **Chief Operating & Financial Officer**	• 25 years; corporate & finance experience	Novelos
Don Joseph, JD **Chief Legal Officer & Secretary**	• 25 years; general counsel & senior management • J.D. University of Texas School of Law	bvgh BIO Ventures for Global Health

Investment Highlights

- Acer's pipeline includes three clinical-stage product candidates:
  - **EDSIVO™ (celiprolol)** for the treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (COL3A1) mutation
  - **ACER-001** (a fully taste-masked, immediate release formulation of sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders (UCDs) and Maple Syrup Urine Disease (MSUD)
  - **Osanetant** for the treatment of various neuroendocrine disorders

- Acer's product candidates are believed to present a comparatively de-risked profile, having one or more of:
  - Favorable safety profile
  - Clinical proof-of-concept data
  - Mechanistic differentiation
  - Accelerated path for development (e.g. Priority Review, 505(b)(2), etc.)

- Multiple key regulatory milestones:
  - ✓ EDSIVO™ acceptance of NDA w/ Priority Review: **December 24, 2018**
    - EDSIVO™ PDUFA action date: **June 25, 2019**
  - Osanetant anticipated IND filing: **H2 2019**
  - ACER-001 (UCD) anticipated NDA submission: **H1 2020**

- Expected to have sufficient capital into H1 2020

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Clinical Pipeline

Program / Indication	Novel MOA / Unique Characteristics	Phase 1	Phase 2	Phase 3	NDA	Market
EDSIVO™ (celiprolol)						
vascular Ehlers-Danlos syndrome (COL3A1+)	Induces vascular dilatation and smooth muscle relaxation					
ACER-001 (reformulated sodium phenylbutyrate)						
Urea Cycle Disorders	Taste-masked formulation; evaluating comparability to Buphenyl®					
Maple Syrup Urine Disease	Inhibition of BCKD kinase to increase BCAA metabolism					
Osanetant						
Neuroendocrine Disorders	Neurokinin 3 Receptor Antagonist					

Overview

Mechanism of Action

- **EDSIVO™ has a unique pharmacological profile**
- β2 and β3 adrenergic receptor agonist; selective β1 and α2 adrenergic receptor antagonist; activates endothelial Nitric Oxide Synthase (eNOS)
- EDSIVO's™ potential beneficial effects in vEDS thought to be through vasodilation, providing more stable hemodynamic conditions that lead to a less fragile arterial wall, and vascular smooth muscle relaxation

Disease

- **Outside surgery, no approved therapeutic options for vEDS patients**
- Autosomal dominant connective tissue disorder of collagen synthesis caused by mutations in the COL3A1 gene for type III procollagen
- Characterized by arterial aneurysms, dissections and/or ruptures
- Median survival in the U.S. is estimated to be 51 years of age

Product Profile

- **Pivotal trial demonstrated a 64% reduction in risk of arterial events**
- EDSIVO™ (celiprolol) showed statistically-significant improvement in event-free survival (EFS) compared to control in vEDS patients (n=53)
- Robust evidence from 17-year registry (n=144) published in JACC

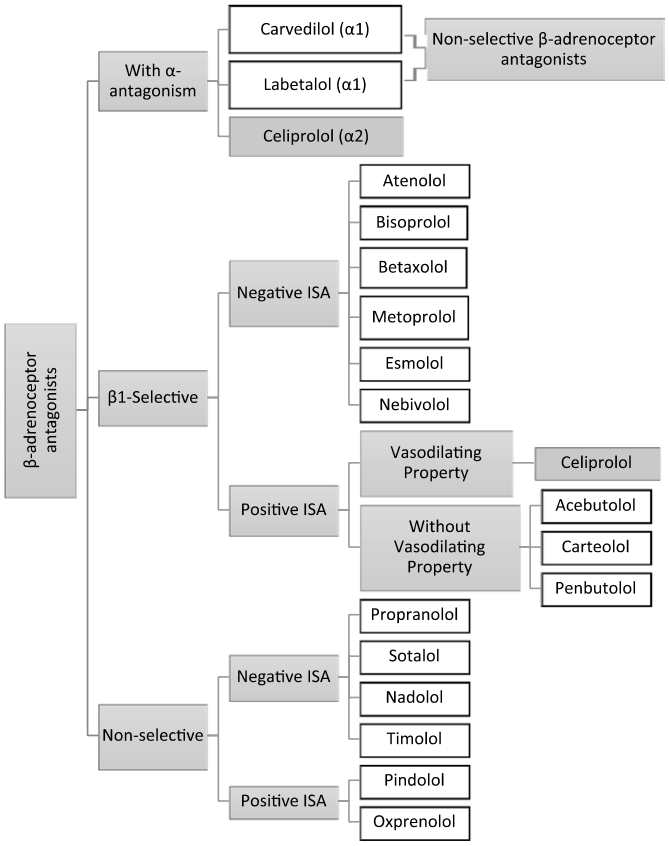
The Opportunity

- **NDA accepted by FDA & priority review granted → PDUFA: June 25, 2019**
- Potentially up to 5,000 COL3A1+ vEDS patients in the U.S.
- Orphan drug designation in vEDS → 7 years market exclusivity
- Reasonable orphan pricing with robust program to support patient access and reimbursement

Unique Mechanism of Action

EDSIVO™ is the only agent to show clinical benefit in patients with vEDS

- EDSIVO™ has a unique pharmacological profile:
  - β2 and β3 adrenergic receptor agonist
  - Selective β1 and α2 adrenergic receptor antagonist
  - Intrinsic sympathomimetic activity (ISA+)
  - Lacks non-specific membrane effects
  - Activates endothelial Nitric Oxide Synthase (eNOS)*
- Void of blood pressure lowering in normotensive people
  - Most vEDS patients are normotensive, thus the potential beneficial effect of celiprolol is unlikely to be through blood pressure lowering (β1 antagonism)
- EDSIVO's™ potential beneficial effects in vEDS patients are thought to be through vasodilation and vascular smooth muscle relaxation, which provides more stable hemodynamic conditions that lead to a less fragile arterial wall



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Cardiology in Review 2017;25: 247–253.

* Hypertension 2003 Nov;42(5):1004-13.

Vascular Ehlers-Danlos Syndrome (vEDS)

- Ehlers-Danlos syndrome (EDS) is a group of hereditary disorders of connective tissue
- vEDS (EDS type IV) is the severe subtype:
  - Characterized by aneurysms, dissections and/or ruptures
    - Vascular
    - Gastrointestinal
    - Uterine
  - Autosomal dominant (50%); spontaneous mutations (50%)
  - Diagnosed by clinical symptoms and confirmed by presence of mutations in the COL3A1 gene
  - Events occur in 25% of patients before the age of 20, but 90% by the age of 40
  - Median age of death is estimated to be 51 years[1]
- No approved therapeutic options for vEDS
  - Current treatment is focused on surgical intervention

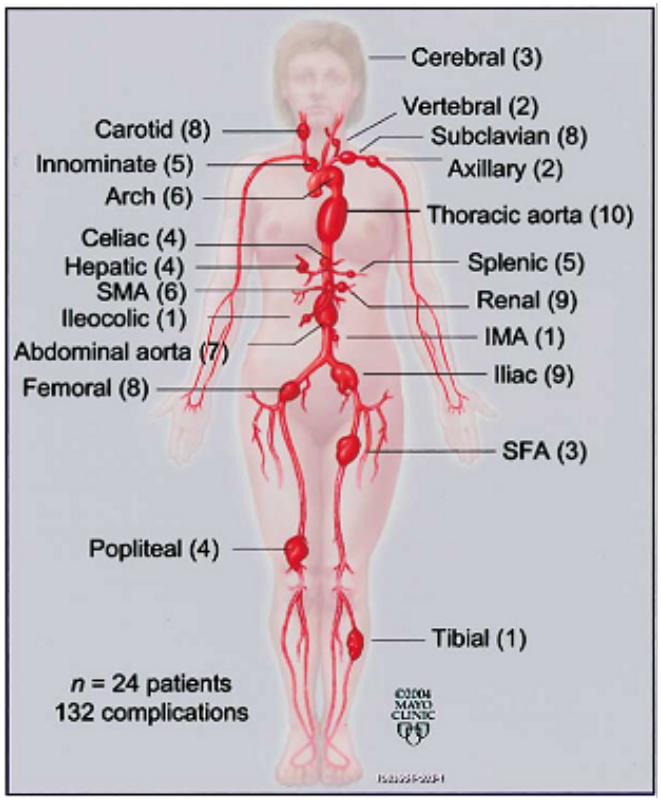

Fig. 3 Distribution of 132 vascular complications in 24 patients with a clinical diagnosis of EDS type IV. J Vasc Surg 2005;42:98-106.

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[1] Pepin Genet Med. 2014 Dec;16(12):881-8.

Pivotal Clinical Trial

Statistically-significant Efficacy:

- Trial stopped early for clinical benefit (mean follow-up 47 months)
- The primary endpoint (arterial dissection or rupture) affected 5 (20%) celiprolol patients and 14 (50%) controls (hazard ratio [HR] 0.36; p=0.04)
- Primary and secondary endpoints (intestinal or uterine rupture) affected 6 (24%) celiprolol patients and 17 (61%) controls (HR 0.31; p=0.01)
- Post-hoc analysis of 33 patients with confirmed COL3A1 mutation indicated equal benefit for the primary (HR 0.24; p=0.04) and secondary endpoints (HR 0.25; p=0.02)
- Author's Comments: "We suggest that celiprolol might be the treatment of choice for physicians aiming to prevent major complications in patients with vEDS"

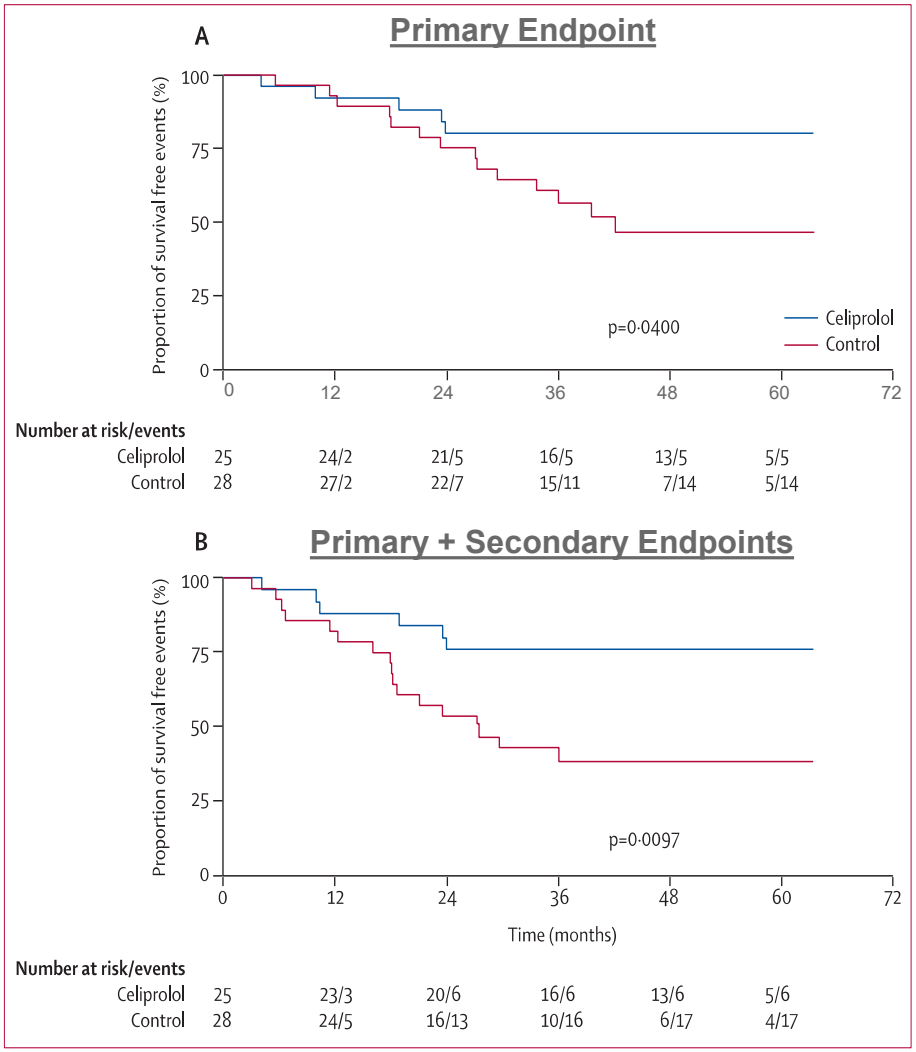

Figure 2: **Kaplan-Meier curves of event-free survival in 53 patients with vascular Ehlers-Danlos**
Primary endpoint (A). Primary and secondary endpoints (B).

Ong Lancet 2010; 376: 1476-84.

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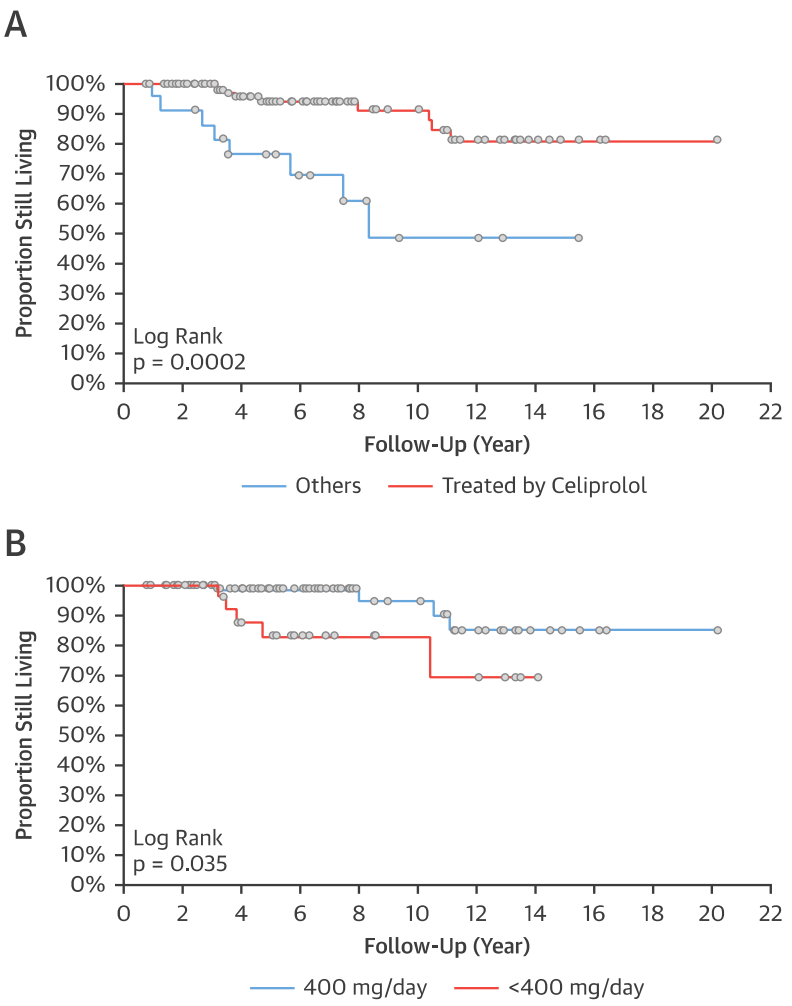
Long-Term Observational Study

Statistically-significant Efficacy:

- Between 2000 and 2017, 144 patients (median age at diagnosis 34.5 years; 100% COL3A1+) were included in this study
- (A) Patients not treated with celiprolol had a significantly worse survival outcome than treated patients:
  - Overall survival was 80.7% (95% CI: 67.8% to 93.6%) in those treated with celiprolol (n = 110) versus 48.5% (95% CI: 19.7% to 77.4%) in those not treated (n = 22) after 11.1 years of follow-up: p = 0.0002
- (B) Survival was significantly improved in patients taking celiprolol 400 mg/day compared with patients taking lower doses, suggesting a dose effect and that 400 mg/day should be considered the optimal treatment dose:
  - At the end of follow-up, survival was 85% (95% CI: 70.5% to 99.5%) in those patients treated with celiprolol 400 mg/day and 69.2% (95% CI: 41.4% to 97.0%) in those taking celiprolol 100 to 300 mg/day: p = 0.035
- Statistically significant decreases in hospitalization rates on intra-patient basis pre-and post-celiprolol treatment

FIGURE 3 Kaplan-Meier Survival Analysis of vEDS Patients in Groups I and II *COL3A1* Pathogenic Variants, According to Celiprolol Treatment



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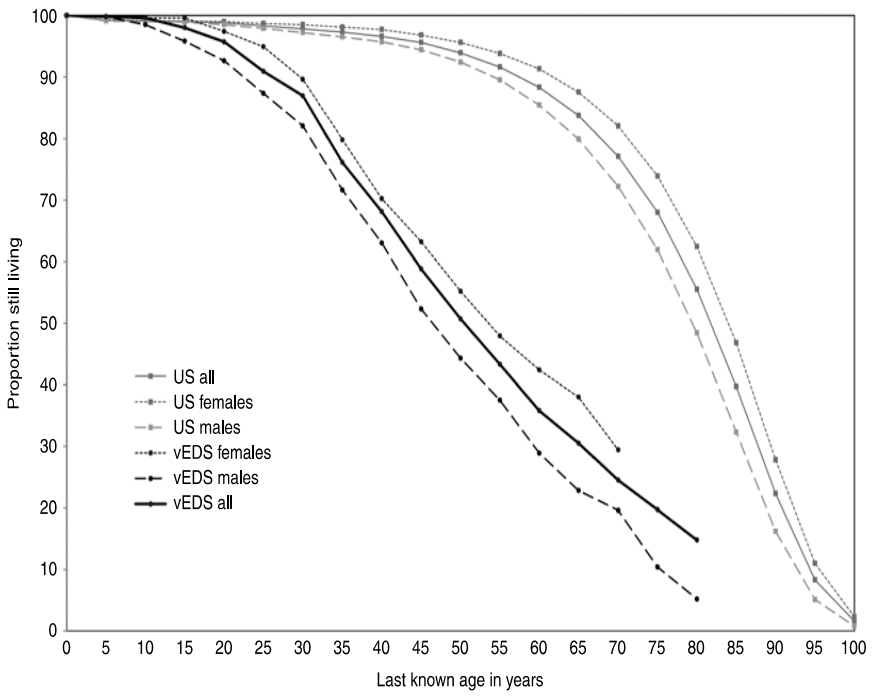
Frank et al., J Am Coll Cardiol. 2019 Apr 23;73(15):1948-1957.

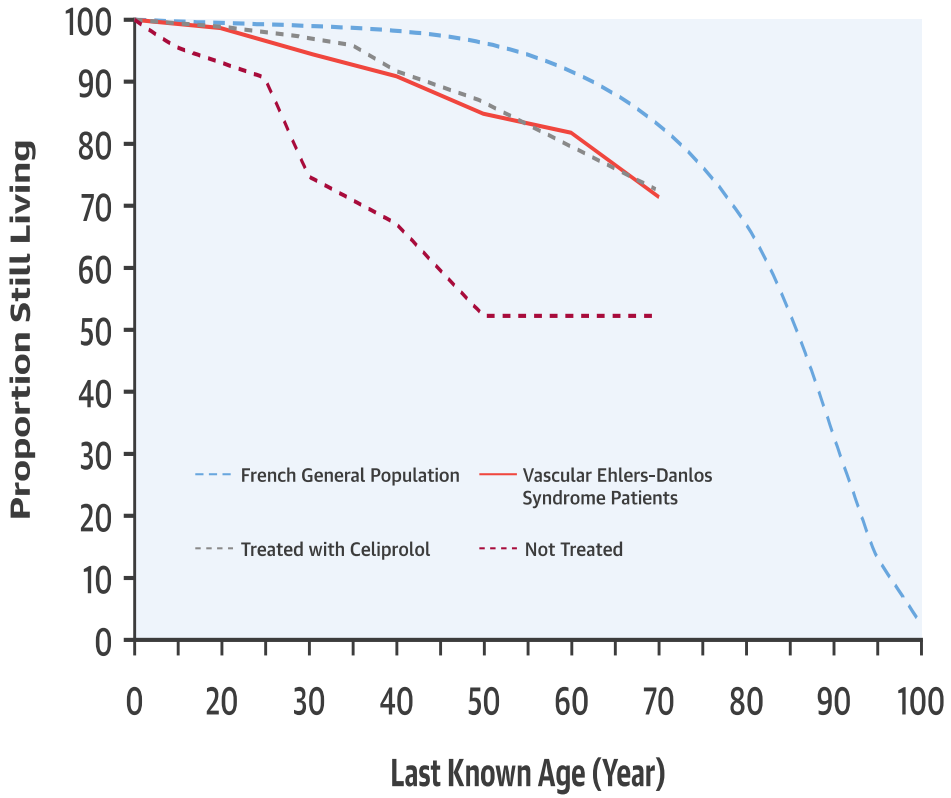
U.S. vs. French vEDS Patients

U.S. vEDS Natural History
1,231 patients (>90% COL3A1+; 630 index / 601 relatives)



Median Survival: 51 years (46 y.o. males; 54 y.o. females)

French vEDS Population
144 patients (100% COL3A1+; 91 index / 53 relatives)



Median Survival: Not Met

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Pepin Genet Med. 2014 Dec;16(12):881-8.
Frank et al., J Am Coll Cardiol. 2019 Apr 23;73(15):1948-1957.

SVM Poster: Antihypertensive Therapy

Background

- No currently approved medications to treat vEDS
- There is no evidence supporting the use of antihypertensive medications in vEDS

Methods

- Retrospective analysis of U.S. insurance claims identifying vEDS patients 2014-2017
- Patient data stratified based on claims for patients taking antihypertensive medications and not taking an antihypertensive medication
- Calculated and compared clinical event rates for each group

Results

- Study suggests that minority of U.S. vEDS patients (34%) are being treated with antihypertensive medications
- Data showed no significant difference in clinical event rates in patients taking antihypertensive medications (16.5%) vs. those not taking an antihypertensive (15.6%) medication
- Underscores need for an effective treatment

Table 4. Rate of Clinical Events in vEDS Patients on Antihypertensive Therapy

vEDS Patient Group	No. Patients (%)	Rate of Clinical Events	*P*-value (vs. No Antihypertensive)
No antihypertension therapy	2,371 (65.6%)	371 (15.6%)	-
Any antihypertension therapy	1,243 (34.4%)	205 (16.5%)	0.51
Beta blocker	895 (24.8%)	146 (16.3%)	0.64
ACE inhibitor	231 (6.4%)	38 (16.5%)	0.75
ARB	228 (6.3%)	55 (24.1%)	0.999
Calcium channel blocker	254 (7.0%)	33 (13.0%)	0.27

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Tetali et al. Poster Presentation: *Pilot Study to Evaluate Effect of Antihypertensive Therapy on the Rates of Clinical Events in Patients with Vascular Ehlers-Danlos Syndrome.* Society for Vascular Medicine 2019 Annual Scientific Sessions, May 30, 2019

Launch Plan*

Patient Identification & Partnerships

Goals:

1. **To identify up to 2,000 confirmed COL3A1+ patients by end of Launch Year**
2. **Expand to up to 5,000 confirmed COL3A1+ patients**

Key Activities:

- Market Sizing
- Genetic Testing Program
- Patient Advocacy

vEDS Network of Excellence (U.S.)

Goals:

1. **To establish a National vEDS Network of Excellence with up to 50 centers at Launch**
2. **Integrated Evidence Generation Strategy**

Key Activities:

- Reference Centers
- KOL Advisory Boards
- Phase 4 Studies, if required
- Investigator Initiated Studies (IISs)

Market Access Ecosystem

Goals:

1. **To ensure optimal pricing and access to all COL3A1+ patients**
2. **Robust HCP & Patient Support Programs**

Key Activities:

- Establish "Acer Care" Patient Support Services
- Payer Engagement Plan
- HCP / Patient Advocacy
- Establish HUB / SPP
- Pharmacoeconomics (HEOR)

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* Commercialization of EDSIVO™ is subject to FDA approval

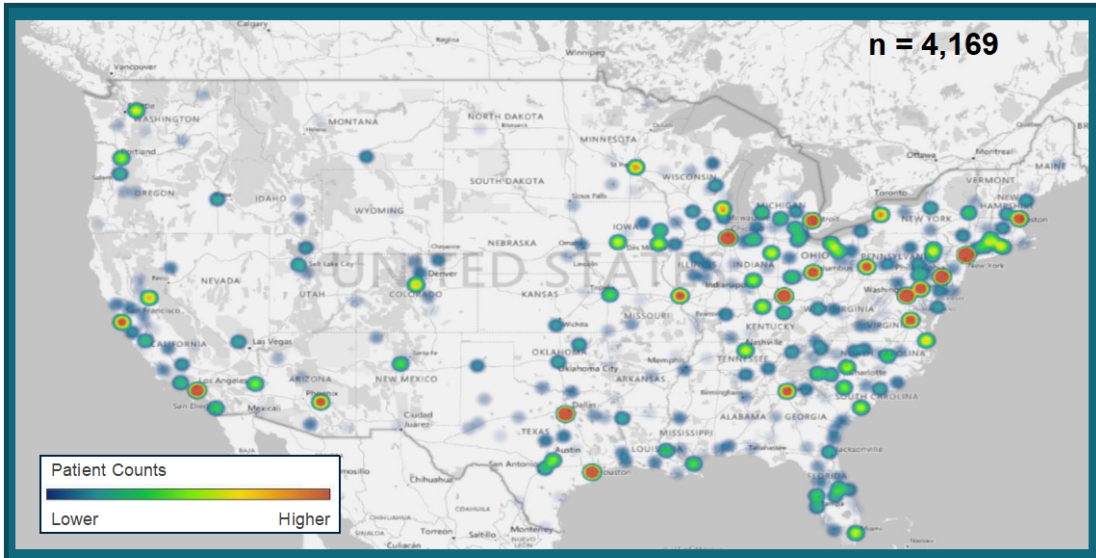
EDSIVO™

Commercial & Medical Affairs Leadership

Matt Seibt **Interim Head of Commercial**	• 22 years; sales, market access & product launch experience • Launched 19 products in primary care, specialty and rare diseases	Biogen VERTEX
Andrew Spaziani **Head of National Sales**	• 28 years; sales leadership exp. in orphan and ultra-orphan markets • Award-winning sales leader, including 6 product launches • Hired 6 Acer sales reps each with 25+ years of experience	BIOMARIN Genentech Retrophin
Srinivas Tetali, M.D. **VP, Medical Affairs**	• 15 years; medical affairs, marketplace and launch experience • M.D. University of Health Sciences/Siddhartha Medical College • Lead on Zonegran, Fragmin, Vyyanse, Kogenate, BeneFIX & Xyntha • Hired Hilary Mandler, Acer's Head of MSLs	Shire REGENERON braeburn
Hilary Mandler, PharmD. **Head of MSLs**	• 15 years; global MSL leadership experience • Hired 3 Acer MSLs • Plan to hire 2 additional MSLs by July	Shire Bristol-Myers Squibb Company

vEDS Market Sizing

- Acer survey of commercial genetic labs for confirmed COL3A1+ vEDS = **~2,000 patients**
- HVH Evaluation (Truven Health MarketScan* = 190M covered lives)*
  - vEDS patient population for basis of market sizing = **4,169 patients**
  - This projects the potential vEDS patients (U.S. population (2018) = 325M) = **~7,100 patients**
  - Need to confirm COL3A1+ status



The raw patient counts are consistent with the larger population areas

Name	vEDS Patients (Rank)
New York-Newark-Jersey City, NY-NJ-PA Metro Area	168 (1)
Chicago-Naperville-Elgin, IL-IN-WI Metro Area	115 (2)
Dallas-Fort Worth-Arlington, TX Metro Area	107 (3)
Washington-Arlington-Alexandria, DC-VA-MD-WV Metro Area	103 (4)
Philadelphia-Camden-Wilmington, PA-NJ-DE-MD Metro Area	98 (5)
Cincinnati, OH-KY-IN Metro Area	89 (6)
Houston-The Woodlands-Sugar Land, TX Metro Area	80 (7)
Los Angeles-Long Beach-Anaheim, CA Metro Area	77 (8)
Detroit-Warren-Dearborn, MI Metro Area	68 (9)
Baltimore-Columbia-Towson, MD Metro Area	61 (10)

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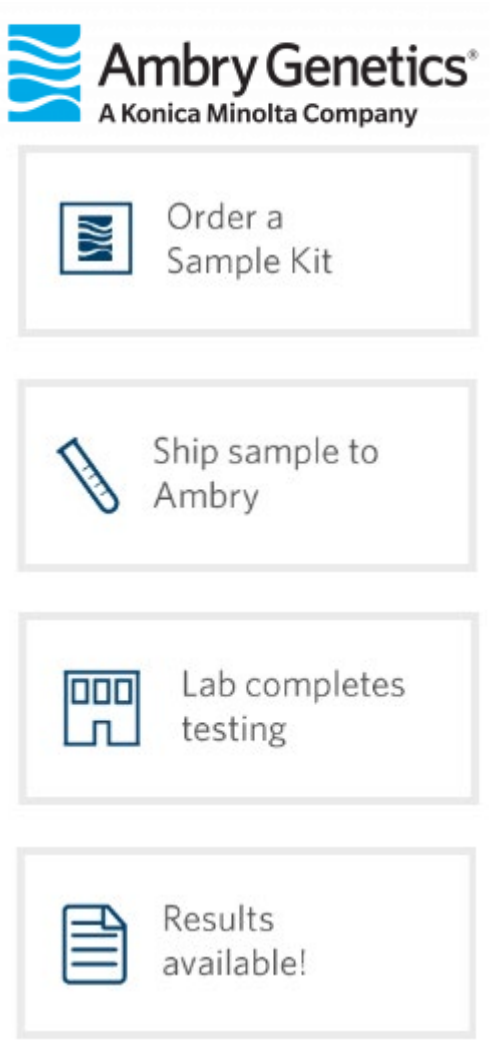
HVH PATIENT PRECISION ANALYTICS

* Study conducted on 06/2017; based on sampling of phenotype, rather than genotype.

Genetic Testing Program (COL3A1)

- Goal: Provide DNA sequencing via NGS or Sanger sequencing (the test should be sufficiently sensitive to detect disease mutation in at least 95% of individuals with COL3A1 mutation)
- Confirm COL3A1+ status of clinically diagnosed patients
- Identify and test family members
- Integrate details into Acer web assets
- Link to third party patient community sites



Patient Advocacy

- Find and engage vEDS patients
- Support community growth
- Create vEDS educational programs
- Promote genetic testing awareness
- Develop / support national vEDS organization

Backpack Health

THE MARFAN FOUNDATION

NORD® National Organization for Rare Disorders

EVERYLIFE FOUNDATION FOR RARE DISEASES

Our Challenge To Find Your Cure — Annabelle's Challenge — AnnabellesChallenge.org

Global Genes® Allies in Rare Disease

Genetic Alliance

Ryan's Challenge

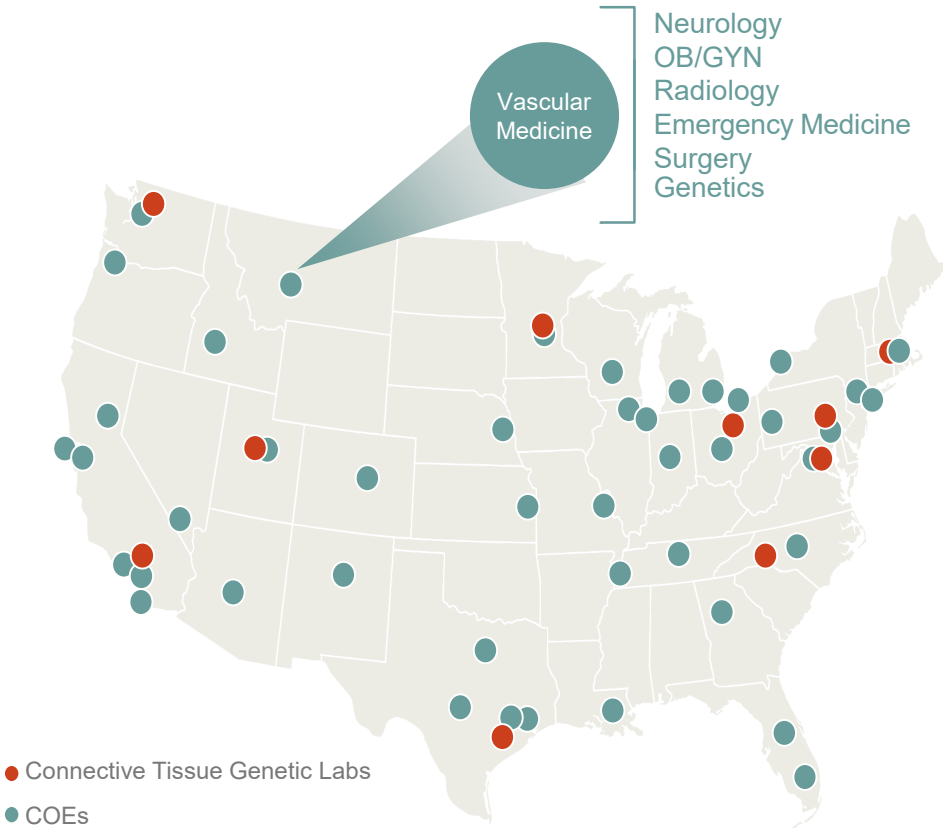
FIGHTveds

The Ehlers-Danlos Society

DEFYFOUNDATION

ThinkGenetic

vEDS Network of Excellence



vEDS Reference Centers

1. **Identify vascular medicine specialists / cardiologists who:**
   - Can manage patients and their families
   - Refer to CV surgeons
   - Work closely with a team of specialists
2. **Expand specialist support team**
   - Care for other aspects of disease management
   - Includes: GI, OB/GYN, Genetics, Surgeons, etc.
3. **vEDS Network of Excellence**
   - 50 Centers at launch
   - Grow to 100 Centers in 3 years
   - ~20-50 vEDS patients per Center
4. **Focused and dedicated medical & commercial effort to support launch**
   - 20-25 field-based support
   - Sales & MSLs

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* Commercialization of EDSIVO™ is subject to FDA approval

EDSIVO™

Market Access Ecosystem

Securing Patient Access Requires Strategic Alignment of Market Access Ecosystem

- Genetic Testing
- Adherence & Persistence Program
- Financial Patient Assistance Program

- Innovative pricing model
- Price predictability
- SAM Compendia strategy

- Dedicated personnel to ensure excellence
- Implement Acer Care & EDSIVO clinical program
- Data aggregation / reporting platform

- EDSIVO Value Proposition
- Build / leverage HCP advocacy with Payers & Policy
- Develop patients' advocacy to HCP & Payers

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Market Opportunity

- If approved, EDSIVO™ will be the only FDA-approved therapy to treat vEDS patients
- Up to 5,000 COL3A1+ vEDS patients in the U.S.
- Establishing vEDS Network of Excellence
  - Focused, dedicated field support (20-25 people)
- Orphan pricing well supported by initial payer research, with additional validation from pharmacoeconomic models
- Provide a robust patient support services program to help patients remove barriers to care
- Granted U.S. Orphan Drug Designation for vEDS (January 2015)
  - If approved, would grant 7 years market exclusivity in vEDS
  - Potential +0.5 years pediatric exclusivity
  - Use patent applications filed may provide additional exclusivity

ACER-001

Overview

Mechanism of Action

- **Small molecule with unique MOAs in various disorders**
- **UCDs**: NaPB is a prodrug of phenylacetate, a NH4+ scavenger
- **MSUD**: NaPB is an allosteric inhibitor of BCKD kinase

Diseases

- **UCDs**: A group of metabolic genetic diseases that lead to toxic build-up of NH4+
- **UCDs**: Currently treated with Ravicti®, Buphenyl®, Ammonul®, and a highly-restricted diet
- **MSUD**: A metabolic genetic disease that leads to toxic build-up of leucine and other branched-chain amino acids
- **MSUD**: Currently managed with a highly-restricted diet; poor compliance

Product Profile

- **A taste-masked, immediate release formulation of sodium phenylbutyrate**
- **UCDs**: Will conduct a PK/BE study to show bioequivalence to Buphenyl
- **MSUD**: POC study suggests ~60% of patients have 30% reduction in Leucine
- Formulation patent filed

The Opportunity

- **Anticipate NDA submission for UCD in H1 2020**
- **UCDs**: >2,000 patients in the U.S.; ~600 patients treated with sodium / glycerol phenylbutyrate
- **MSUD**: ~800 eligible patients in the U.S.
- Reasonable orphan pricing with robust program to support patient access and reimbursement

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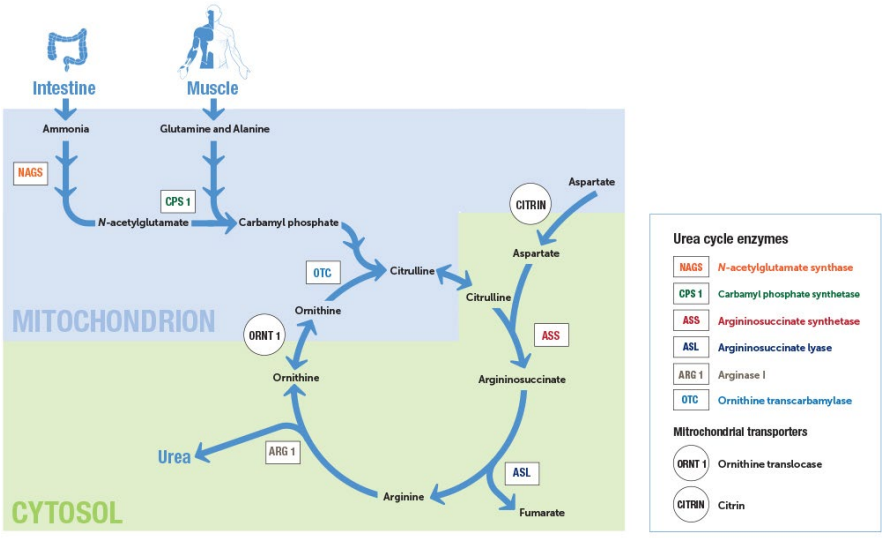
Urea Cycle Disorders (UCDs)

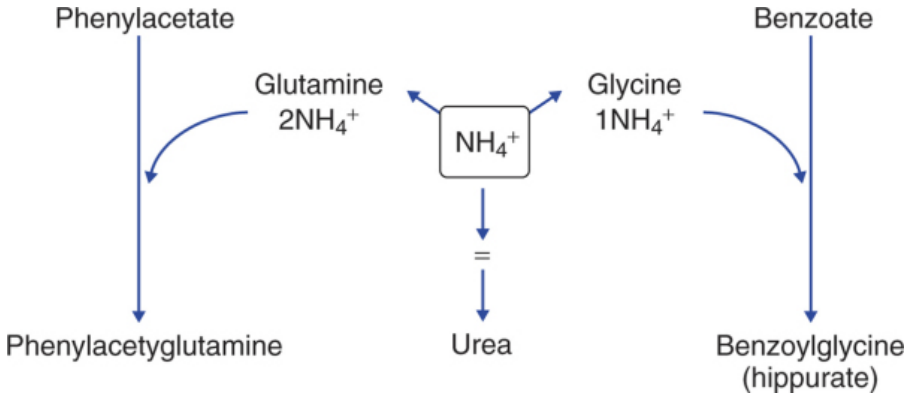
- Urea cycle disorders are a group of genetic disorders caused by mutations that result in a deficiency of one of the six enzymes in the urea cycle. These enzymes are responsible for removing ammonia from the blood stream

- The estimated incidence of urea cycle disorders is 1 in 35,000 births[1]

- Treatment options for UCDs include:
  - Phenylbutyrate (Buphenyl®, Ravicti®)
  - IV Benzoate / Phenylacetate (Ammonul®)
  - Sodium Benzoate
  - Restricted Diet
  - Liver Transplantation



The Urea Cycle

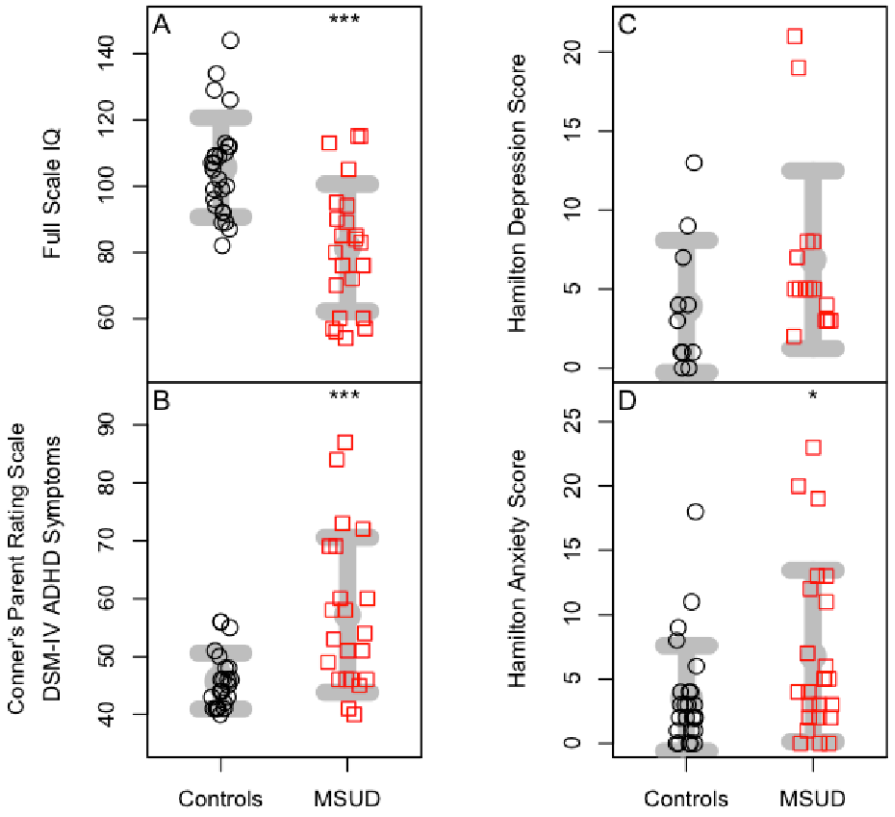


Mechanism of ammonia diversion from the urea cycle with the administration of sodium phenylacetate, sodium benzoate, or sodium phenylbutyrate (a prodrug of phenylacetate)

[1] GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2018. 2003 Apr 29 [updated 2017 Jun 22].

Maple Syrup Urine Disease (MSUD)

- MSUD is an inborn error of Branched-chain Amino Acid (BCAA) – leucine, isoleucine, valine – metabolism
  - Caused by deficiency of the mitochondrial Branched-chain Keto Acid Dehydrogenase complex (BCKDC)
  - ~800 patients in U.S., ~3,000 patients worldwide
  - MSUD Family Support Group has >500 patients
  - Part of newborn screening in U.S., UK, Germany
- High leucine levels lead to chronic and acute neurological damage
  - Lower IQ
  - Mental impairment (poor cognitive function)
  - Social impairment (poor executive function)
  - Metabolic decompensation (seizures and coma)
- A highly-restricted diet is the primary treatment
  - Consists of BCAA-free synthetic foods and formula
  - Very few foods have low BCAAs (fruits & vegetables)
  - Balancing act: enough BCAAs for growth & development



indicates $p < 0.05$, *** $p < 0.001$ compared to the control group*

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* Muelly 2011 Neuropsychiatric and Neurochemical Sequelae of MSUD.

Lower BCAAs in UCDs & MSUD

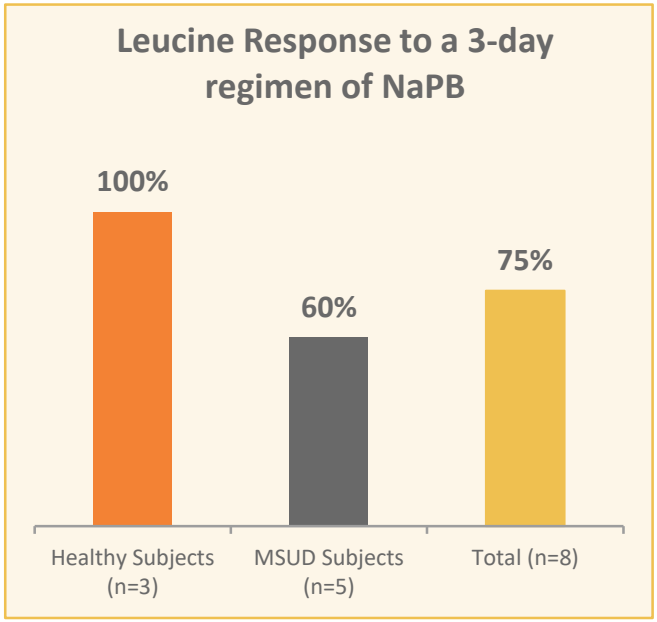
- In addition to lowering levels of ammonia in patients with urea cycle disorders (UCDs), sodium phenylbutyrate (NaPB) also significantly reduces branched-chain amino acids (BCAAs)[1]

- NaPB's ability to lower leucine and other BCAAs could provide clinically-meaningful benefit in another genetic disease: Maple Syrup Urine Disease (MSUD)[2]
  - Open label pilot study at BCM – 3 healthy and 5 MSUD subjects with late onset disease
  - Despite the short treatment duration (3 days) NaPB showed statistically significant (intra-subject) reduction in leucine in 75% of the subjects

Retrospective study of BCAA levels in 553 UCD patients treated with NaPB or without

Table 2
Branched chain amino acid levels in patients taking NaPBA (n = 212) vs. patients not taking NaPBA (n = 341). The medians and interquartile ranges are provided for each BCAA. The p-value has been corrected for multiple testing using the Bonferroni correction.

	NaPBA	No NaPBA	$p_{corrected}$ value
Leucine (μMol/L)	60 (40–85)	95 (72–121)	<0.005
Valine (μMol/L)	128 (92–169)	176 (142–217)	<0.005
Isoleucine (μMol/L)	31 (22–49)	49 (36–65)	<0.005



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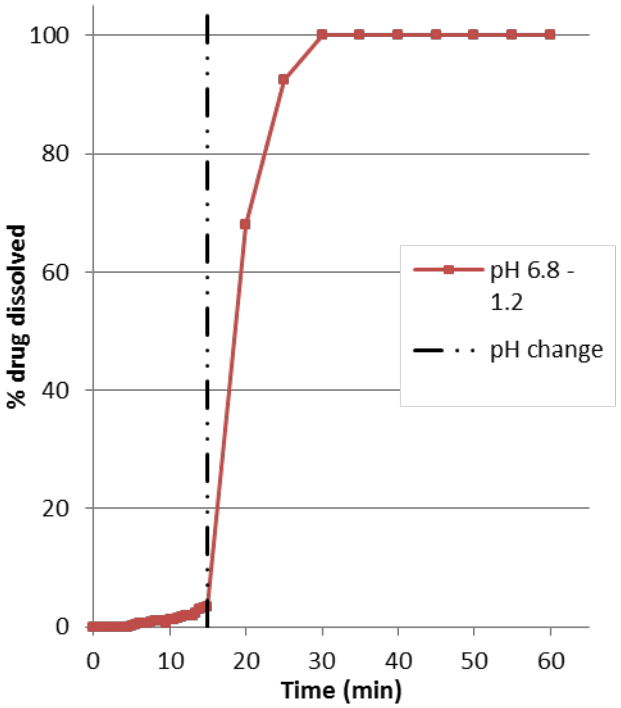
[1]Burrage et al., Mol. Genet. Metab. 2014.
[2]Brunetti-Pierri et al., Hum Mol Genet. 2011 February 15; 20(4): 631–640.

Taste-Masked, Immediate Release Formulation

Mouth → Stomach



Excellent protection for several minutes at mouth pH followed by rapid release at stomach pH

Phenylbutyrate Formulations			
	ACER-001*	RAVICTI®	BUPHENYL®
Efficacy/Safety in UCDs	✓	✓	✓
Efficacy/Safety in MSUD	✓	✗	✓
Palatability/Compliance	✓	✓	✗
Reasonable Orphan Pricing	✓	✗	✓

- Acer is working closely with KOLs & patient advocacy groups to provide a compelling alternative treatment option for patients with UCDs
- ACER-001 provides significant differentiation from other approved formulations of phenylbutyrate
- Ravicti® / Buphenyl® reported 2018 revenue $248.4M (U.S. / UCDs only)**
  - Ravicti® annual price: ~ $800K pppy
  - Buphenyl® annual price: ~ $150K pppy

*Subject to FDA Approval
**Ravicti & Buphenyl pppy is based on patient weight.

Clinical Development

- IND: Active as of May 30th, 2019
- Pivotal Bridging Study: initiation expected in Q4'19
- Study Design:
  - 36-64 healthy subjects
  - 2-day trial
  - 1 dose of Buphenyl, 1 dose of ACER-001
  - Primary Endpoint: PK (bioequivalence)
  - Secondary Endpoint: Taste assessment survey
- EAP: assuming BE, aim to initiate expanded access program by early 2020

Market Opportunity

- Target existing sodium phenylbutyrate market share in UCDs
  - 2018 U.S. Revenue for Ravicti® & Buphenyl® = $248.4M (~600 patients)
  - Well differentiated formulation
  - Will be competitively priced
- Life cycle expansion opportunity in MSUD
- Barriers to entry:
  - Filed formulation patent application (January 2016)
  - Issued patent (US/EP) “Methods of modulation of branched chain acids and uses thereof”
  - UCDs: 505(b)(2) Exclusivity: 3 years market exclusivity
  - MSUD: Granted U.S. Orphan Drug Designation for MSUD: 7 years market exclusivity
  - Pediatric Exclusivity: 6 months
- Provide a robust PAP to help offset costs to patients

Overview

Mechanism of Action

- **Osanetant is a selective, non-peptide tachykinin NK3 receptor antagonist**
- NK3R is the main receptor for neurokinin B (NKB), a tachykinin peptide primarily found in the arcuate nucleus (ARC) of the hypothalamus

Diseases

- **NKB/NK3R is implicated in a variety of human functions and affects the hypothalamus-pituitary-gonadal axis (HPG axis)**
- The HPG axis plays a critical part in the development and regulation of a number of the body's systems, such as the reproductive and immune systems
- Clinical data with other NK3R antagonists have demonstrated statistically significant improvement in vasomotor symptoms and polycystic ovarian syndrome (PCOS)

Product Profile

- **Clinical and laboratory safety results are available from 21 completed Phase I and II studies in a total of 1,586 people, of whom 665 patients were treated with osanetant**
- Oral bioavailability, readily crosses the blood-brain barrier

The Opportunity

- **Company developing the only other NK3R antagonist in clinical development (Phase 2a) acquired by Astellas in April 2017 for up to €800M**
- Several disorders involving the HPG axis could benefit from treatment with NK3R antagonist
- Anticipate filing IND in H2 2019

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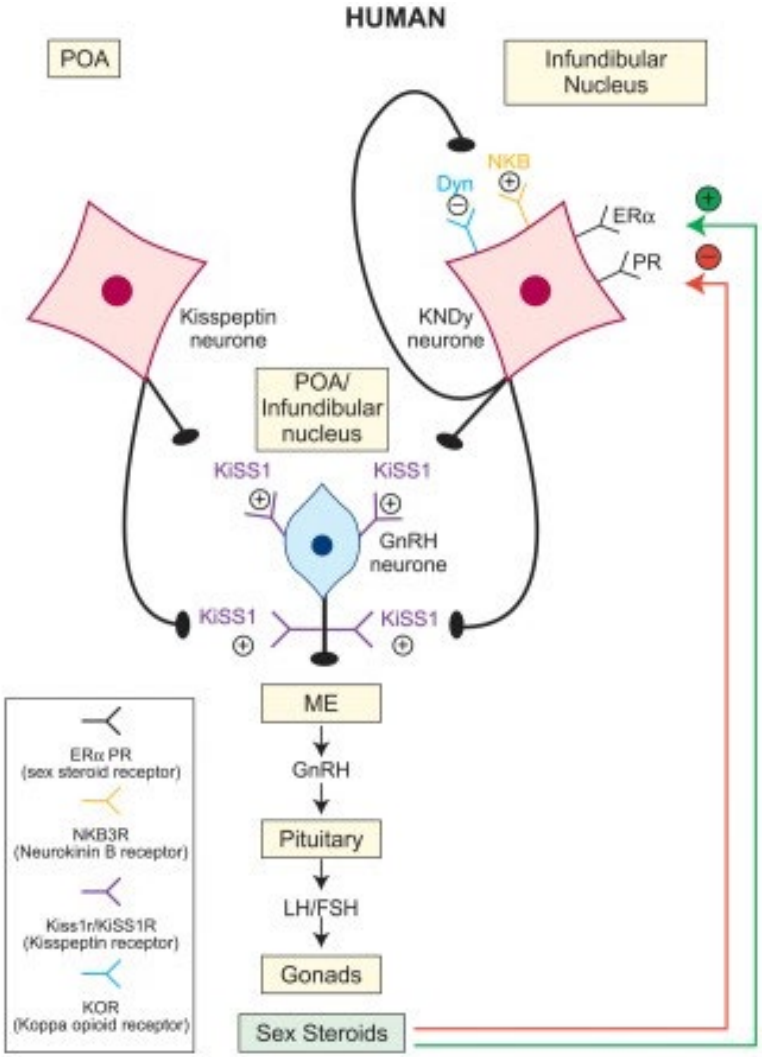
* Am J Psychiatry. 2004 Jun;161(6):975-84.

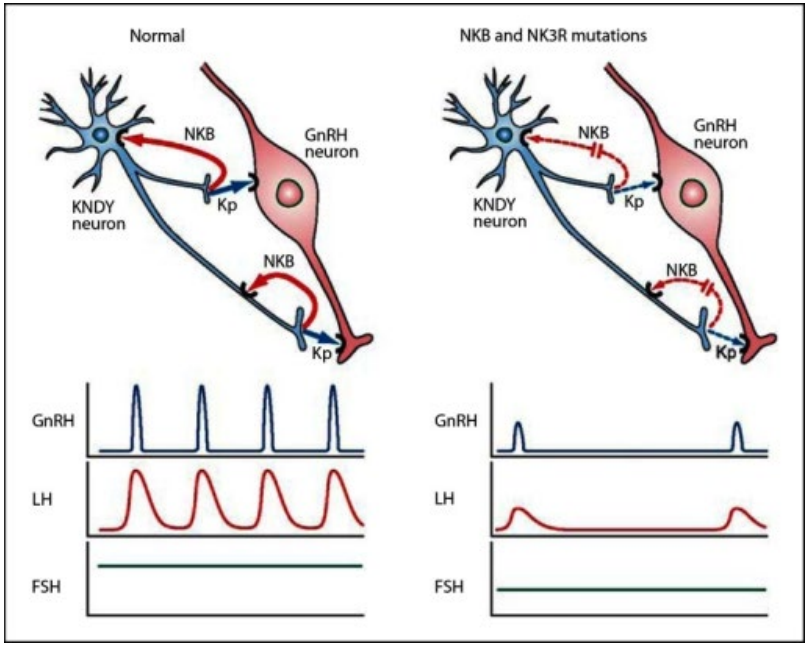
NK3 Receptor (Neurokinin B)

- Neurokinin B (NKB) belongs to a group of neuropeptides, called tachykinins or neurokinins, that includes substance-P (SP), neurokinin A (NKA), and two N-terminally extended forms of NKA, neuropeptide g and neuropeptide K
- The biological effects of tachykinins are mediated through specific receptors denoted NK1, NK2, and NK3
- NKB is the preferred endogenous ligand of tachykinin NK3 receptors
- The tachykinin NK3 receptors are located primarily in the brain, while a few receptors are also present in the peripheral nervous system (intestines, placenta)



Role of NKB/NK3R in Neuroendocrinology

- Neurokinin B (NKB), a member of the tachykinin-peptide family, has emerged as an important modulator of reproductive function
- This appears to be mediated, at least partially, by its ability to auto-regulate the activity of Kiss1 neurons in the ARC
- The NKB system may play a more prominent stimulatory role on the gonadotropic axis during early stages of sexual maturation
- Recent studies demonstrated hypogonadotropic hypogonadism in patients bearing inactivating mutations in TAC3 or TACR3 genes, encoding NKB and its receptor NK3R, respectively



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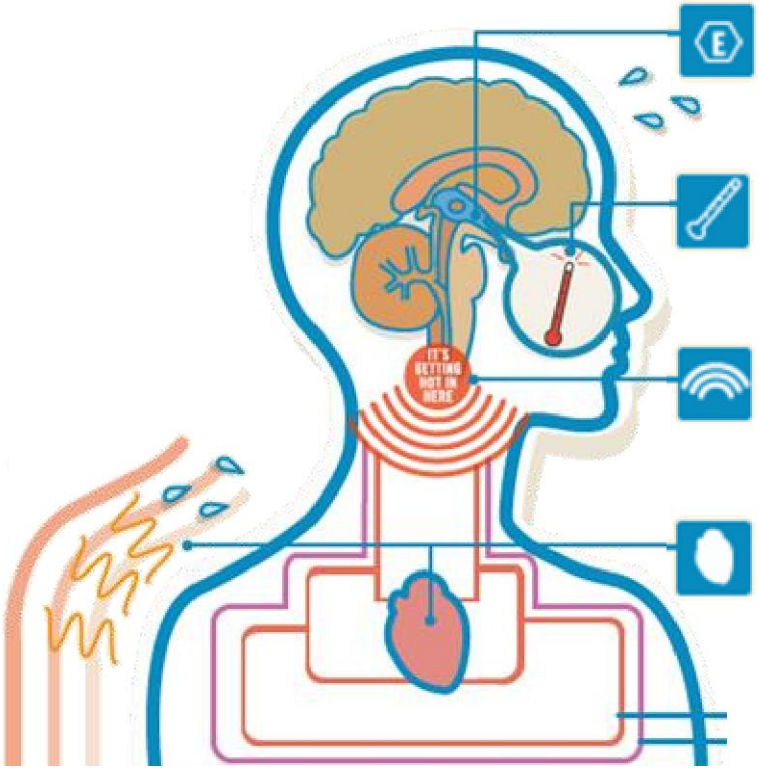
Neuroendocrinology. 2013;97(2):193-202.

Mechanism of Vasomotor Symptoms (VMS)

- Vasomotor symptoms, typically comprised of hot flashes and night sweats, are associated with decreases in reproductive hormones commonly associated with menopause (e.g. MR-VMS)



A diminished amount of hormones, such as estrogen, affects the hypothalamus

↓

This confuses the hypothalamus and makes it read "too hot"

↓

The brain responds by relaying an alert to cool off

↓

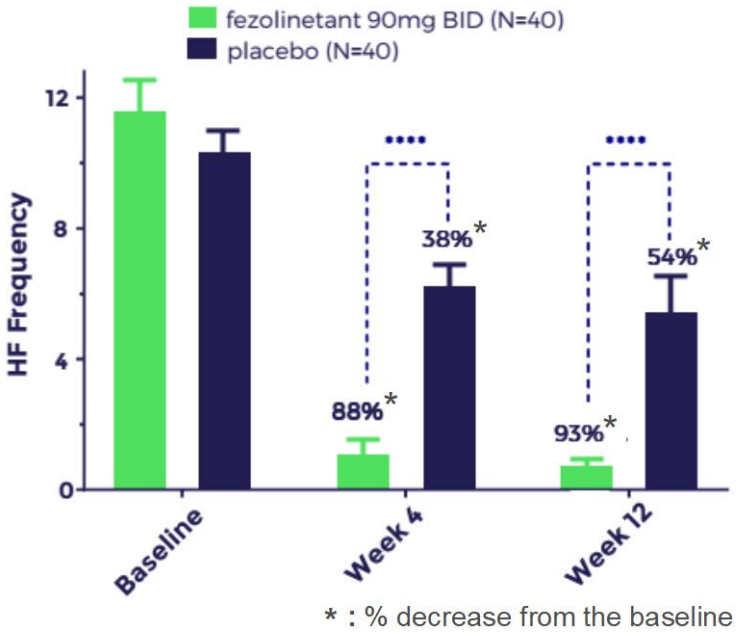
The body then tries to cool off by beginning to perspire

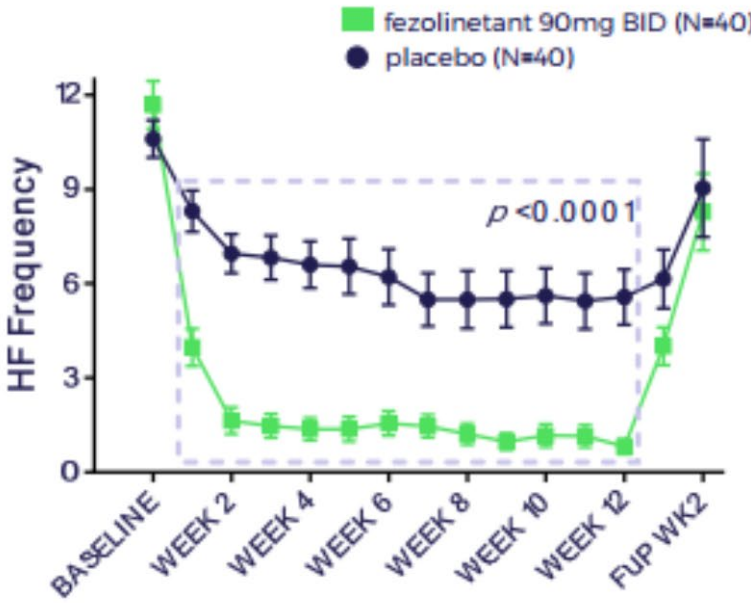
The Oncologist, Oct 2003, vol.8 no.5 474-487.
http://www.everydayhealth.com/menopause/science-behind-the-hot-flash.aspx

NK3R Antagonist Clinical POC in VMS

- Fezolinetant is a NK3R antagonist, originally developed by Ogeda SA
- Ogeda was acquired by Astellas in 2017 for up to €800M

Average Daily Hot Flash Frequency Reported as per FDA Guidance





At Week 4:

- *fezolinetant group: 14/40 patients have ZERO hot flash*
- *placebo group: 2/40 patients have ZERO hot flash*

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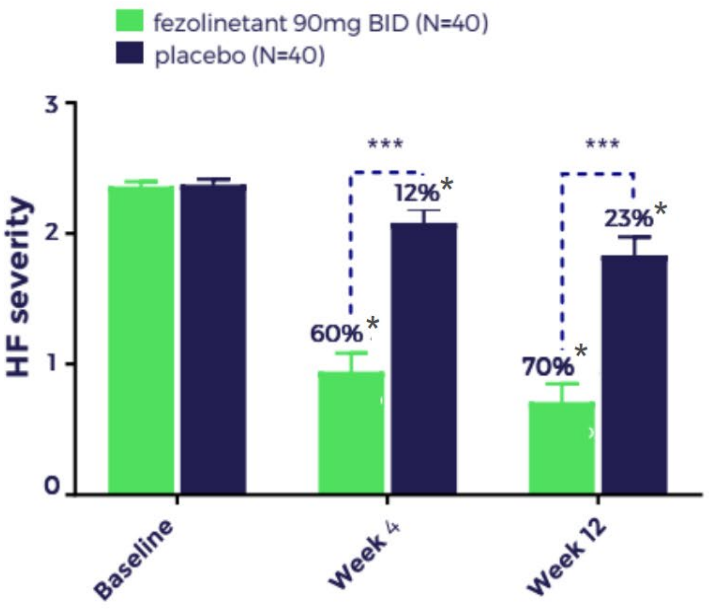
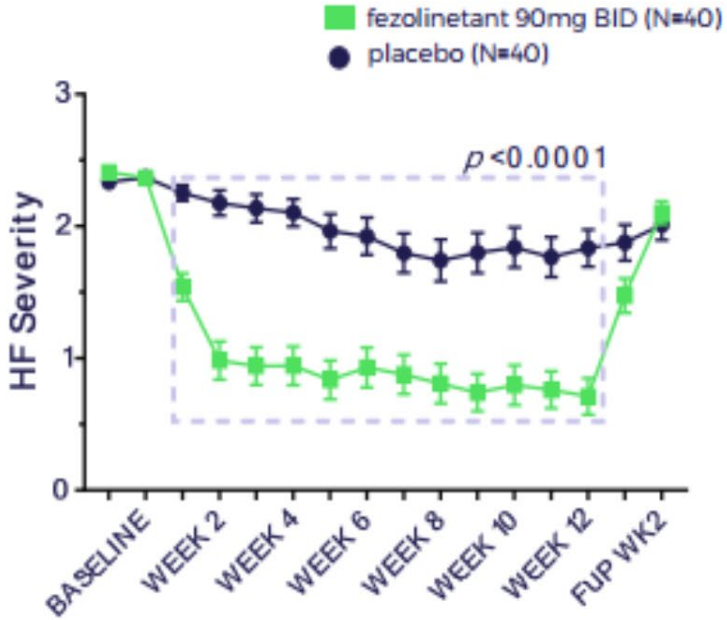
Herman Depypere et al., ENDO2017

NK3R Antagonist Clinical POC in VMS

- Fezolinetant is a NK3R antagonist, originally developed by Ogeda SA
- Ogeda was acquired by Astellas in 2017 for up to €800M

Score of average severity of Hot Flash, irrespective of frequency of Hot Flash





* : % decrease from the baseline

1 - Mild: sensation of heat without sweating
2 - Moderate: heat with sweating, but able to continue activity
3 - Severe: heat with sweating, causing cessation of activity

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Herman Depypere et al., ENDO2017

Market Opportunity

- Acer acquired worldwide rights to osanetant from Sanofi in January 2019
- Clinical proof of concept studies with other NK3R antagonists have demonstrated rapid and clinically meaningful improvement in vasomotor symptoms and PCOS
  - Ogeda SA was acquired by Astellas in 2017 for up to €800M
- Osanetant was studied in healthy subjects and schizophrenic patients (n=~1,500) with clinical and laboratory safety data from 21 completed Phase 1 and 2 studies
- Refining development plan with top opinion leaders
- Multiple rare and life-threatening neuroendocrine disorders will be explored in multiple phase 1/2 studies, evaluating pk/pd and safety
- Anticipate filing IND in H2 2019

Financial Overview

- Cash:
  - $31.8M as of March 31, 2019
  - Expected to have sufficient capital into H1 2020
- Capitalization as of March 2019:
  - 10.1M shares of common stock outstanding
  - 11.3M shares of common stock fully diluted
- $87M invested through August 2018
  - Top institutional investors include (as of 3/31/19):
    - TVM, Nantahala, Vivo, Bukwang Pharmaceutical, Driehaus, Vanguard, Heartland, Acuta, Emerald, Avego

Summary

- Acer's pipeline includes three clinical-stage product candidates:
  - **EDSIVO™ (celiprolol)** for the treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (COL3A1) mutation
  - **ACER-001** (a fully taste-masked, immediate release formulation of sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders (UCDs) and Maple Syrup Urine Disease (MSUD)
  - **Osanetant** for the treatment of various neuroendocrine disorders

- Acer's product candidates are believed to present a comparatively de-risked profile, having one or more of:
  - Favorable safety profile
  - Clinical proof-of-concept data
  - Mechanistic differentiation
  - Accelerated path for development (e.g. Priority Review, 505(b)(2), etc.)

- Multiple key regulatory milestones:
  - ✓ EDSIVO™ acceptance of NDA w/ Priority Review: **December 24, 2018**
    - EDSIVO™ PDUFA action date: **June 25, 2019**
  - Osanetant anticipated IND filing: **H2 2019**
  - ACER-001 (UCD) anticipated NDA submission: **H1 2020**

- Expected to have sufficient capital into H1 2020