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

Corporate Presentation

March 2020

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, timelines, future financial position, future revenues, projected expenses, regulatory submissions, actions or 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 capital 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 of any of our product candidates in any target indication and any territory; the adequacy of our capital to support our future operations and our ability to successfully initiate and complete clinical trials and regulatory submissions; our progress toward possible approval for EDSIVO™ in light of the Complete Response Letter we received June 2019 and the Formal Dispute Resolution Request response letter received March 2020; the ability to protect our intellectual property rights; our strategy and business focus; and the development, expected timeline 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, our ability to reduce our operating expenses and conserve cash on a net basis as a result of our prior or any future corporate restructuring initiative, 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, the substantial costs and diversion of management's attention and resources which could result from pending securities litigation, risks related to the drug development and the regulatory approval process, including the timing of regulatory actions, 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 significant unmet medical needs

- Headquartered: **Newton, MA**
- Headcount: **18**
- Founded: **December 2013**
- Public: **September 2017**
- Cash: **$12.1M** as of December 31, 2019
  - Expected to have sufficient capital to fund current operations through end of 2020, excluding:
    - Support for EDSIVO™ development and precommercial activities
    - Planned osanetant clinical trial

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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
Harry Palmin **Chief Operating & Financial Officer**	• 25 years; corporate & finance experience	Novelos
Matt Seibt **Chief Commercial Officer**	• 22 years; sales, market access & product launch	Biogen
John Klopp **Chief Technical Officer**	• 18 years; orphan manufacturing & commercialization	BIOMARIN
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 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 induced Vasomotor Symptoms (iVMS) where Hormone Replacement Therapy (HRT) is likely contraindicated

- 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
  - Potential expedited paths for development through specific FDA-established programs

- Multiple anticipated key regulatory milestones:
  - ✓ EDSIVO™ FDRR[1] appeal denied but exploring possible paths forward: **Q1 2020**
  - ✓ ACER-001 (UCD) pivotal BE trial completion: **Q1 2020**
  - Osanetant IND submission: **2H 2020**
  - Osanetant Initiate Phase 1/2 PK/PD/safety trial[$]: **End 2020**
  - ACER-001 (UCD) NDA submission*[$]: **Q1 2021**

- Expected to have sufficient capital through end of 2020, excluding support for EDSIVO™ development and precommercial activities and planned osanetant clinical trial

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[1]Formal Dispute Resolution Request
[$]Subject to additional capital
*Assuming successful outcomes of additional nonclinical work and 12-month long-term stability data

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Clinical Pipeline

Program / Indication	Novel MOA / Unique Characteristics	Development Status
EDSIVO™ (celiprolol)		
vascular Ehlers-Danlos syndrome (COL3A1+)	Induces vascular dilatation and smooth muscle relaxation	**TBD***
ACER-001 (taste-masked, immediate-release form of sodium phenylbutyrate)		
Urea Cycle Disorders	Taste-masked formulation; evaluating bioequivalence to BUPHENYL® **	**Pre-NDA**
Maple Syrup Urine Disease	Inhibition of BCKD kinase to increase BCAA metabolism	**Phase 2**
Osanetant		
Induced Vasomotor Symptoms (iVMS)	Neurokinin 3 Receptor Antagonist	**Phase 1/2**

*Complete Response Letter received June 2019; Formal Dispute Resolution Request submitted to the FDA December 2019; response received March 2020 denying appeal of the Complete Response Letter but describing possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVO™ NDA
**Pivotal bioavailability and bioequivalence (BE) trial

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EDSIVO™ Overview

Disease Overview

- **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

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 vascular dilatation and smooth muscle relaxation, thereby reducing the mechanical stress on collagen fibers within the arterial wall

Product Profile

- **BBEST Clinical Trial: 64% reduction in risk of arterial events observed[1]**
- Statistically-significant improvement in event-free survival (EFS) compared to control in vEDS patients (n=53)[1]

The Opportunity

- **FDRR response appeal denied but exploring possible paths forward**
- Evaluating possible next steps with the goal of resubmission of the EDSIVO™ NDA
- Neither resubmission nor the prospect of approval of EDSIVO™ NDA is assured

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[1]Ong Lancet 2010; 376: 1476-84.

EDSIVO™: Regulatory Timeline

- **June 2019**: Received CRL from FDA
  - CRL stated it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS
- **December 2019**: Submitted Formal Dispute Resolution Request (FDRR) to the Office of New Drugs (OND)
- **March 2020**: Received OND FDRR response
  - Denied appeal of CRL
  - OND described possible paths forward for Acer to explore that could provide substantial evidence of effectiveness needed to support a potential resubmission of NDA
  - Evaluating possible next steps with the goal of EDSIVO™ NDA resubmission (neither resubmission nor approval is assured)
- Updates to be provided as appropriate and the company may discontinue the process at any point where risk/benefit no longer justifies continued resources

FDA: Substantial Evidence of Effectiveness

THE QUANTITY OF CLINICAL EVIDENCE TO ESTABLISH EFFECTIVENESS

Two adequate and well-controlled clinical investigations[2]

- In many situations FDA requires two adequate and well-controlled trials to establish effectiveness
- This reflects the need for substantiation of experimental results

One adequate and well-controlled investigation plus confirmatory evidence[2]

- Under certain circumstances and consistent with FDAMA, FDA can conclude that one adequate and well-controlled clinical investigation plus confirmatory evidence is sufficient to establish effectiveness

One adequate and well-controlled investigation[1]

- FDA can accept a single adequate and well-controlled trial when the results are highly persuasive such that the single trial provides support comparable to that from two adequate and well controlled studies

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1 FDA Guidance Document (1998) 'Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products'
2 FDA Guidance Document (2019) 'Demonstrating Substantial Evidence of Effectiveness for Human Drug and Biological Products'

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
    - Hollow Organs (e.g. 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, and 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

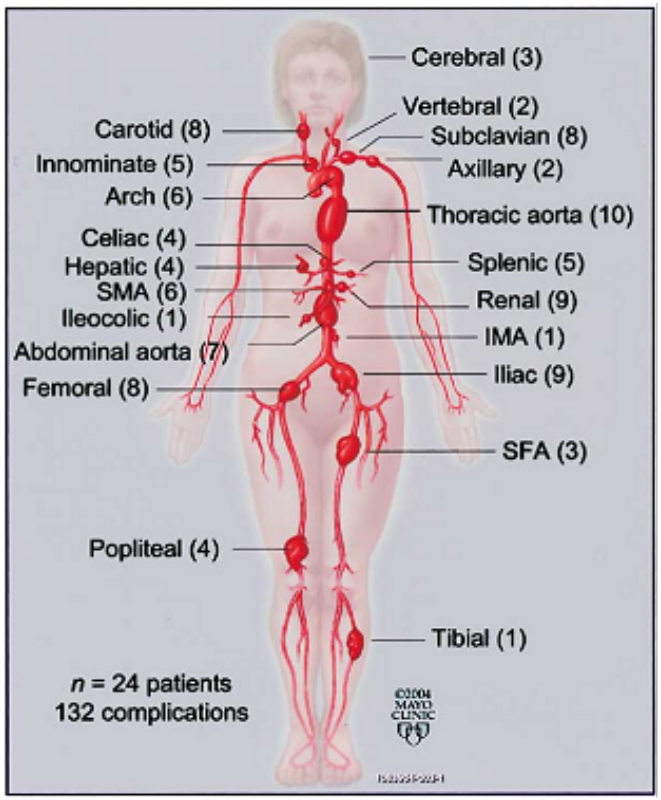

Fig. 3 Distribution of 132 vascular complications in 24 patients with a clinical diagnosis of EDS type IV. J Vasc Surg 2005;42:98-106.

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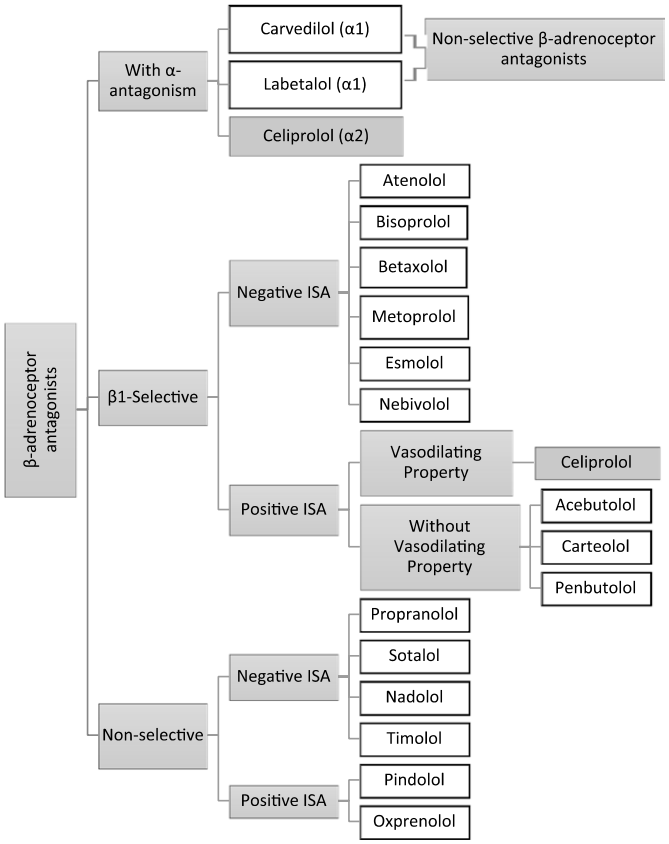
[1]Pepin Genet Med. 2014 Dec;16(12):881-8.

Unique Mechanism of Action

- 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 vascular dilatation and smooth muscle relaxation, thereby reducing the mechanical stress on collagen fibers within the arterial wall



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Cardiology in Review 2017;25: 247–253.
*Hypertension 2003 Nov;42(5):1004-13.

BBEST Trial: COL3A1+ Subpopulation

Efficacy:

- 76% reduction in the risk of fatal or nonfatal cardiac or arterial events in COL3A1+ EDSIVO™ patients vs. control group over mean follow-up of 47 months

- 75% reduction in risk of primary (cardiac or arterial events) and secondary (intestinal or uterine rupture) events in COL3A1+ EDSIVO™ patients vs. control group

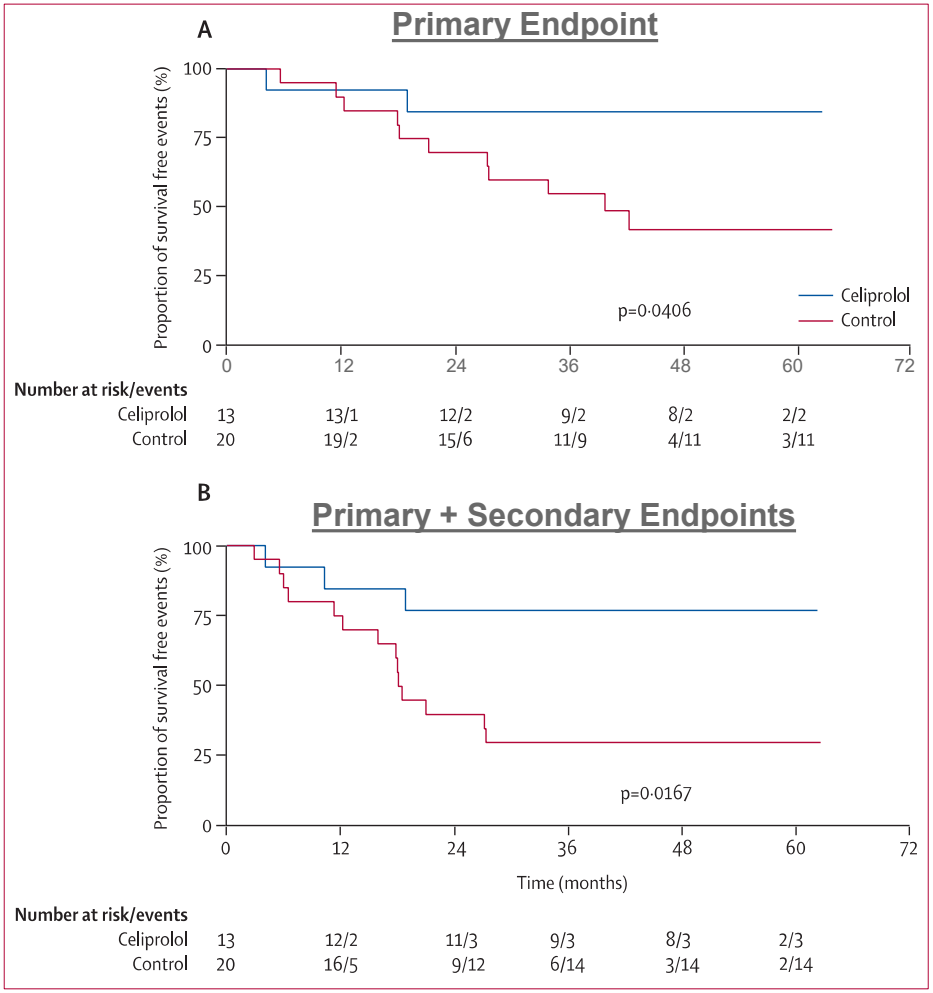

Figure 3: **Kaplan-Meier curves of event-free survival in 33 patients with positive *COL3A1* mutation**
Primary endpoint (A). Primary and secondary endpoints (B).

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Ong Lancet 2010; 376: 1476-84.

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

Disease Overview

- **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**: Trial showed ACER-001 bioequivalence to BUPHENYL®
- **MSUD**: POC study[1] suggests ~60% of patients have 30% reduction in Leucine

The Opportunity

- **Anticipate NDA submission for UCD Q1 2021*$**
- **UCDs**: >2,000 patients in the U.S.; ~600 patients treated with sodium / glycerol phenylbutyrate
- **MSUD**: ~800 eligible patients in the U.S.
- Advantageous orphan pricing with robust program to support patient access and reimbursement

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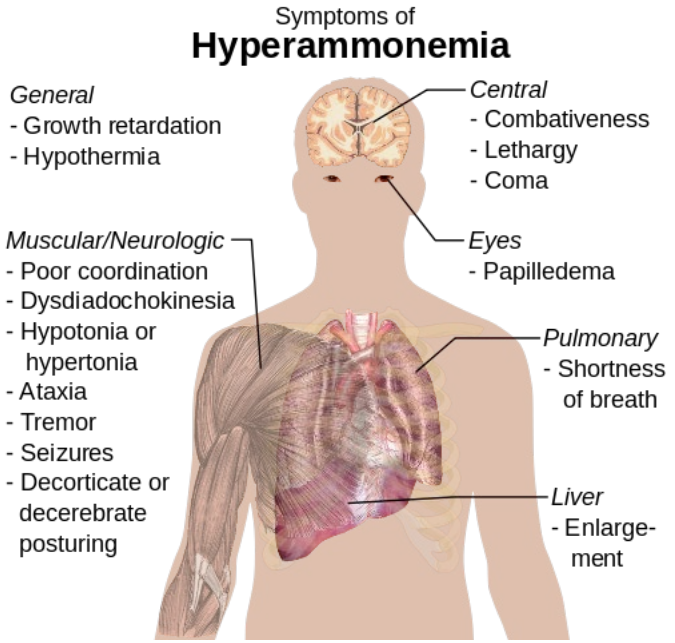
[1]Brunetti-Pierri et al., Hum Mol Genet. 2011 February 15; 20(4): 631–640
*Assuming successful outcomes of additional nonclinical work and 12-month long-term stability data
$Subject to additional capital

UCDs: Clinical Manifestations

- Newborns with severe urea cycle disorders become catastrophically ill with symptoms that mimic sepsis -- failure to feed, lethargy, respiratory distress, seizures and ultimately coma
- Children and adults with milder (or partial) urea cycle enzyme deficiencies may go years without a diagnosis, until a trigger -- a high protein meal, viral illness, excessive exercise or calorie deficiency -- causes excessive ammonia to be produced in the body, resulting in critical elevations of blood ammonia levels
- For individuals with OTC deficiency, typical neuropsychological complications include developmental delay, learning disabilities, intellectual disability, attention deficit hyperactivity disorder (ADHD), and executive function deficits



Reproduced from: http://upload.wikimedia.org/wikipedia/commons/7/76/Symptoms_of_hyperammonemia.svg.

Ah Mew N, et al.. Urea cycle disorders overview. Gene Reviews. Seattle, Washington: University of Washington, Seattle; 1993.
Lichter-Knoecki U., et al. Ornithine Transcarbamylase Deficiency, Gene Reviews. Seattle, Washington: University of Washington, Seattle; 1993-2019.

UCDs: Unmet Need

- **BUPHENYL®**: Foul odor and foul/bitter taste; considered unpalatable*
  - 64% of patients reported it is difficult to take because of taste
  - Physicians reported that 25-33% of patients were prescribed less than target dose due to tolerability
  - Only 25% of patients indicated that they never miss a dose
  - 46% of patients reported taste as the reason for discontinuation*

- **RAVICTI®**: Mostly Tasteless/Odorless
  - Pricing has risen to levels considered challenging
  - Reports of difficult access, unaffordability, and forced switches back to sodium phenylbutyrate
    - For example: BUPHENYL® and RAVICTI® both recently removed from CVS/Caremark formulary for JPMorgan Chase plan members, effective 8/1/2019**
  - Patient groups and physicians have called for a taste-masked, affordable and accessible treatment***

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*Shchelochkov et al., Barriers to drug adherence in the treatment of urea cycle disorders: Assessment of patient, caregiver, and provider perspectives. Molecular Genetics and Metabolism Reports 8 (2016) 43-47.
**https://www.caremark.com/portal/asset/Formulary_Drug_Removals_JPMC.pdf
***Acer Market Research

ACER-001: Differentiation

Phenylbutyrate Formulations			
	ACER-001*	RAVICTI®	BUPHENYL®
Efficacy / Safety in UCDs	✓	✓	✓
Palatability / Compliance	✓	✓	✗ **
Pricing (Per Patient Per Year)	TBD, likely near BUPHENYL	$158k-$1.2M***	$204k-$402k***
Formulation	Multi-Particulate (Sachet)	Oil (Tablespoons)	Powder/Tablets (up to 40 tablets/day)

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*Subject to FDA Approval
**Molecular Genetics & Metabolism Reports 8 (2016) 43-47
***Ravicti & Buphenyl pppy is based on patient weight and WAC price

UCDs: Clinical & Regulatory Path

- Bioavailability and bioequivalence (BE) trials:
  - Part A: single-center, single-blind, randomized, single-dose crossover study designed to evaluate bioavailability of three different oral suspension formulations of ACER-001 compared to BUPHENYL® in 20 healthy adult subjects
    - ✓ Successfully completed and optimal formulation of ACER-001 identified
  - Part B: single-center, single-blind, randomized, single-dose crossover study to demonstrate bioequivalence of the optimal formulation of ACER-001 (chosen from Part A) compared to BUPHENYL® in 36 healthy adult subjects
    - ✓ Successfully completed in Q1 2020
- Taste Assessment trials:
  - Taste assessment of three different formulations of ACER-001 (multi-particulate powder) assessed relative to BUPHENYL® (powder) using certified taste-testers
    - ✓ Successfully completed and informed selection of optimal formulation of ACER-001
- NDA: Anticipate submission Q1 2021[$] pending successful outcome of additional nonclinical work and 12-month long-term stability data

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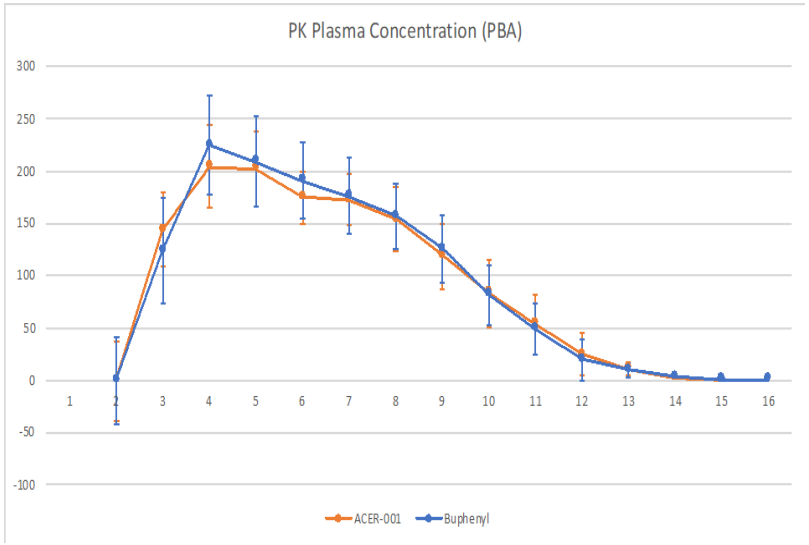
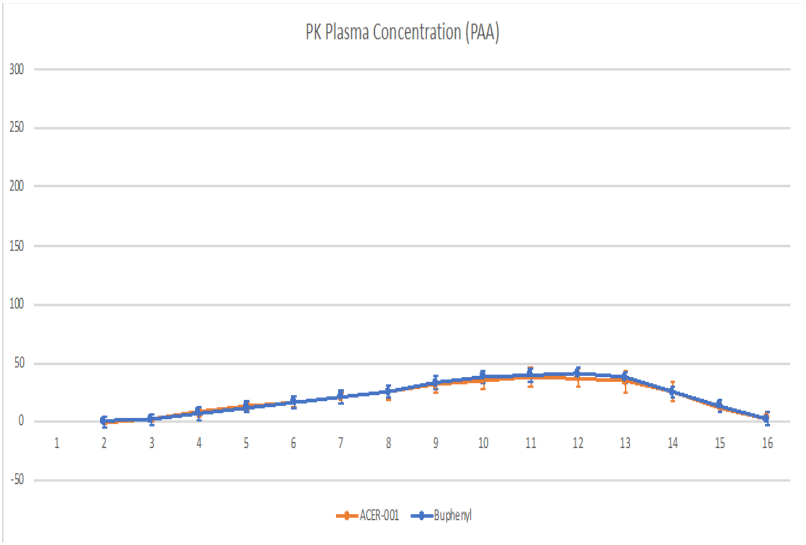
[$]Subject to additional capital

UCDs: Bioequivalence Trial Results (Part A)





Lab Name	Parameter	Geometric Mean Difference	Lower 90% Confidence Limit	Upper 90% Confidence Limit
PBA	Cmax	98.01	93.85	102.36
	AUCt	98.87	96.33	101.47
	AUCinf	98.85	96.32	101.45
PAA	Cmax	97.51	92.82	102.44
	AUCt	95.94	90.35	101.87
	AUCinf	95.16	88.92	101.84

PBA = phenylbutyrate; PAA = phenylacetate

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UCDs: Market Opportunity

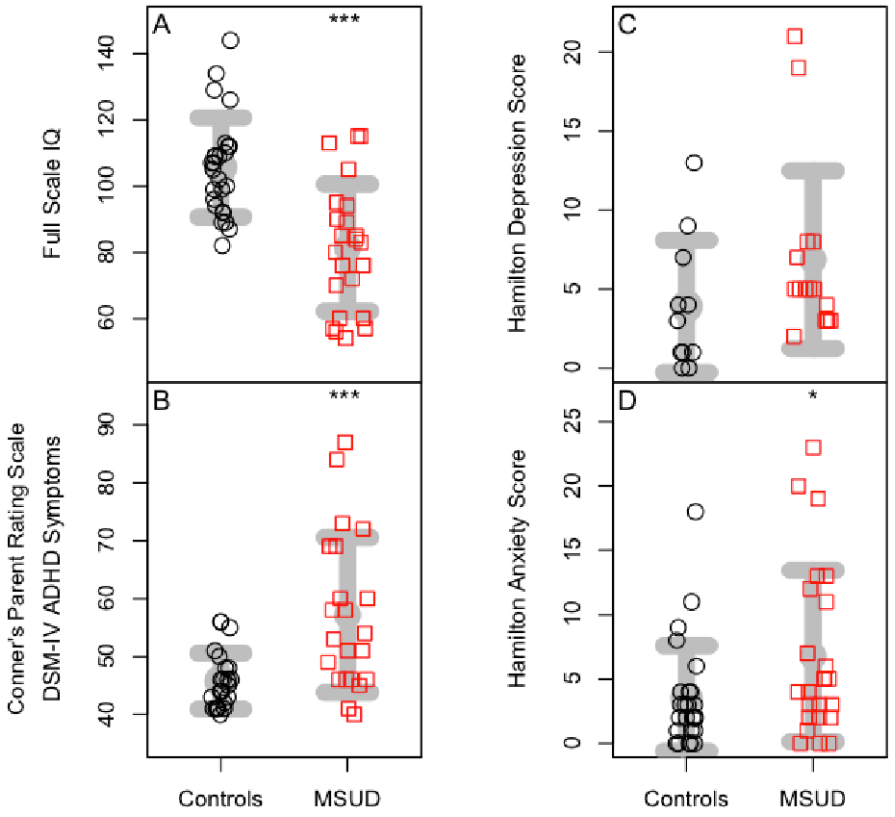
- Target existing Rx market share in UCDs
  - Currently 1,100 patients diagnosed with ~600 patients on Rx therapy*
  - 2018 U.S. revenue for RAVICTI® & BUPHENYL® = $248.4M
  - Goal: transition patients from RAVICTI® & BUPHENYL® to ACER-001 and capture a portion of new UCDs Rx

- "Transition" Value Story: A cost-effective, taste masked alternative for UCDs (assuming successful studies and FDA approval):
  - Bioequivalence to BUPHENYL®
  - Greater compliance/adherence compared to BUPHENYL® expected due to differentiated formulation providing taste masked alternative
  - Competitively priced vs. RAVICTI®
  - Payer engagement strategy to support switching

*https://www.sec.gov/Archives/edgar/data/1386858/000119312515110284/d899054dex992.htm (HZNP IR Presentation)

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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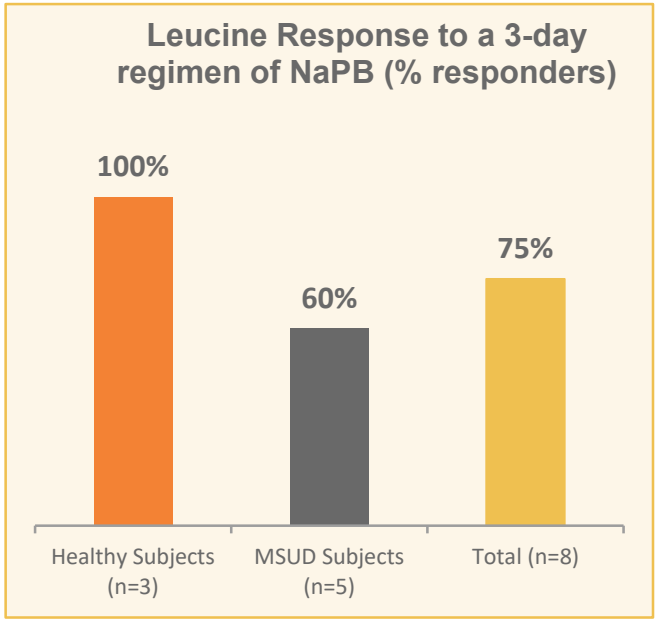
*Muelly 2011 Neuropsychiatric and Neurochemical Sequelae of MSUD.

MSUD: Clinical POC Study

- Design: Open label pilot study[1] at BCM – 3 healthy and 5 MSUD subjects with late onset disease
  - 3 days of steady-state protein diet*; then 3 days of NaPB + diet*
  - BCAAs and BCKAs determined at day 3 of each study period (4 time points)
- Results: NaPB demonstrated statistically significant leucine reduction in all 3 healthy subjects ($p < 0.05$) and 3 out of 5 MSUD patients ($p < 0.05$ in responders)
  - ~30% reduction (28-34%) in leucine in MSUD responders
  - Clinicians view >20-30% ↓ as clinically meaningful**
- Comments: Despite the short treatment duration (3 days) NaPB showed statistically significant (intra-subject) reduction in leucine in 75% of the subjects



Brunetti-Pierri et al., Hum Mol Genet. 2011 February 15; 20(4): 631–640

[1]Brunetti-Pierri et al., Hum Mol Genet. 2011 February 15; 20(4): 631–640
*All subjects received a constant protein intake of 0.6 g/kg/day as combination of BCAA-free formula and whole protein
**Acer commissioned market research

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MSUD: Market Opportunity

- About 1,000 MSUD patients in the U.S., ~3,000 WW*
  - 20-25% MSUD patients in U.S. are Mennonite; incidence up to 1/380
  - Ashkenazi Jewish population; incidence of 1/26,000
- No treatments currently approved for MSUD
- Early treatment may help reduce the rate of neuropsychological comorbidities and optimize growth**
- MSUD specialists recognize NaPB's potential effectiveness, yet tolerability is a concern***
- Plan to initiate Phase 2 trial in MSUD by end of 2020$

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*https://www.ncbi.nlm.nih.gov/books/NBK1319/
**Molecular Genetics and Metabolism Reports 15 (2018)
***Acer Therapeutics: US Market Research – 2014
$Subject to additional capital

ACER-001: Exclusivity / IP

- Filed formulation patent application (filed Oct. 2016)
- Issued patents (US/EP): “Methods of modulation of branched chain acids and uses thereof” [US PATENT NO. 10,092,532] in MSUD
  - Exclusive license rights from Baylor College of Medicine
- UCDs: 505(b)(2) application - 3 years potential market exclusivity from FDA approval
- MSUD: Granted U.S. Orphan Drug Designation: 7 years market exclusivity from FDA approval
- Pediatric exclusivity: +6 months added (if pediatric indication study approved)

Osanetant: 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

Disease Overview

- **iVMS: Induced Vasomotor Symptoms where Hormone Replacement Therapy (HRT) is likely contraindicated**
  - Induced vasomotor symptoms (iVMS) are well documented with the use of hormonal cancer therapies and certain surgical procedures
  - Symptoms such as hot flashes can appear immediately and be severe
  - Traditional HRTs are usually contraindicated

Product Profile

- **Clinical and laboratory safety results are available from 21 completed Phase 1 and 2 studies (325 healthy subjects and 665 patients were treated with osanetant)**
- Oral bioavailability, readily crosses the blood-brain barrier

The Opportunity

- **Acer licensed worldwide rights to osanetant from Sanofi in January 2019**
- Anticipate submitting IND in 2H 2020
- Multiple potential orphan opportunities
- Currently no other NK3R antagonists in development in iVMS space

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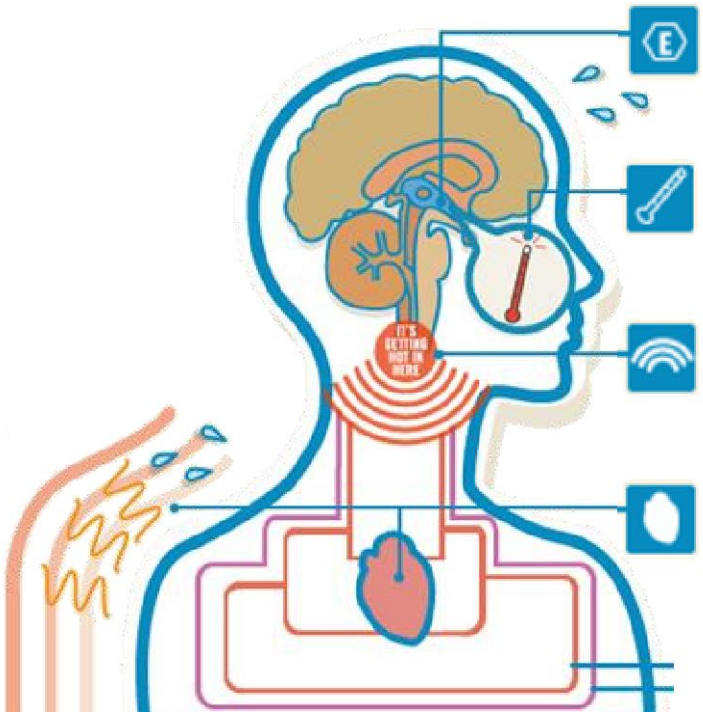
*Am J Psychiatry. 2004 Jun;161(6):975-84

History

- Acer acquired worldwide rights to osanetant from Sanofi in December 2018
- Osanetant (SR142801) was the first selective non-peptide tachykinin NK3 receptor antagonist evaluated as a potential treatment for schizophrenia
- Clinical and laboratory safety results are available from 21 completed Phase 1 and 2 studies in which 325 healthy subjects and 665 schizophrenic patients were treated with osanetant
- No major safety concerns identified from these studies after single-dose and repeated-dose administration of up to 400 mg QD for up to 21 days, and 200 mg QD for up to 6 weeks
- In March 2005, Sanofi-Aventis discontinued the development of osanetant citing 'lack of efficacy compared with placebo' in this indication as a major reason for this decision

Vasomotor Symptoms (VMS): Overview

- VMS, 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

- While VMS associated with menopause can often be treated with hormone replacement therapy (HRT), there are patients who experience VMS who are not in menopause and for whom HRT is likely contraindicated

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

Induced Vasomotor Symptoms (iVMS)

Women who are BRCA+ and have prophylactic bilateral salpingo-oophorectomy (PBSO)

- 67% of women have symptoms of menopause such as hot flashes[5]
- Up to 35% complain of "extremely bothersome" symptoms up to two years after their surgery[6]

Men with HR+ Prostate Cancer (CaP) receiving Leuprolide

- 80% of men experience hot flashes[3]
- 15-27% of patients consider hot flashes the most distressing side effect
- 30-40% experienced moderate-to-severe symptoms
- 20% discontinued or disrupted treatment

Women with HR+ Breast Cancer (CaB) receiving Tamoxifen

- 84% of women experienced hot flashes[1]
- 80% experienced night sweats
- 60% experienced severe symptoms
- Symptoms persisted throughout 5 years of treatment and were mainly attributed to tamoxifen
- After 4.5 years, 46% of women had discontinued tamoxifen[2]

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[1]Moon, Z. et al., JOURNAL OF PSYCHOSOMATIC OBSTETRICS & GYNECOLOGY, 2017 VOL. 38, NO. 3, 226–235
[2]Nichols, H, et al., JNCI J Natl Cancer Inst, 2015, 1–8
[3]Challapalli, A, et al., Clinical and Translational Radiation Oncology 10 (2018) 29–35
[4]Abildgaard, J, et al., JNCI Cancer Spectrum, 2018, Vol. 0, No. 0
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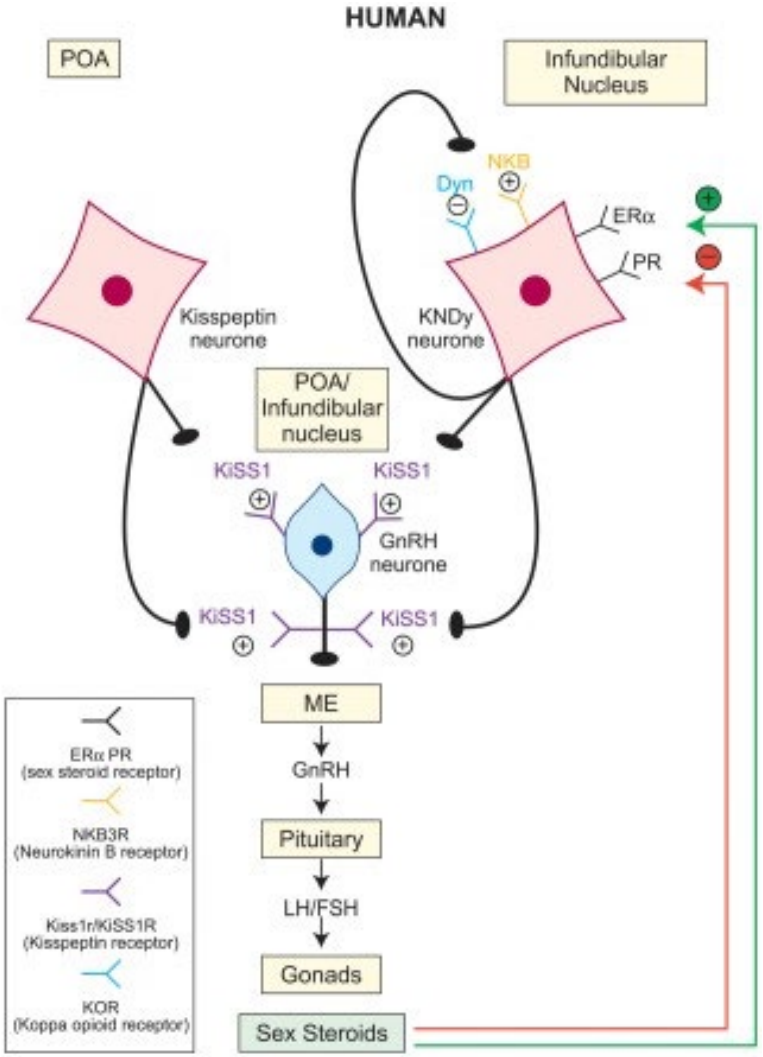
iVMS: The Unmet Need

- Induced vasomotor symptoms (iVMS) are well documented with the use of hormonal cancer therapies and certain surgical procedures
- Symptoms such as hot flashes can appear immediately and be severe
- Traditional HRTs are usually contraindicated
- Non-adherence to therapy can be associated with side effects which increases the mortality risk or shortens the time to recurrence
- A non-hormonal treatment for iVMS is needed to help ensure breast or prostate cancer patients can start and stay on critical hormonal cancer therapy and BRCA2 post-PBSO can obtain help with significantly impactful and limiting iVMS

NK3 Receptor (Neurokinin B)

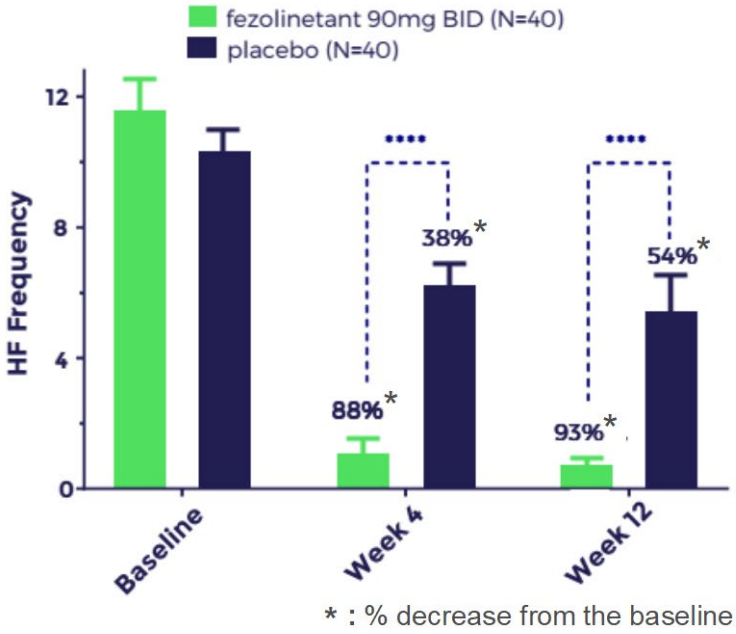
- Neurokinin B (NKB) belongs to a group of neuropeptides, called tachykinins or neurokinins, that includes substance-P (SP), neurokinin A (NKA), and two N-terminally extended forms of NKA, neuropeptide g and neuropeptide K
- The biological effects of tachykinins are mediated through specific receptors 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)

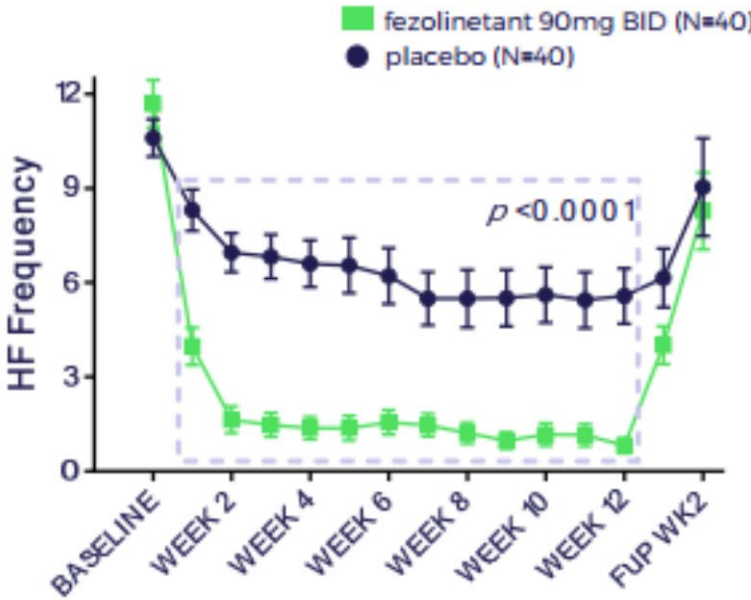


NK3R Antagonist Clinical POC in VMS

- Fezolinetant is a NK3R antagonist being developed by Astellas

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

- Pavinetant (MLE4901) was a NK3R antagonist that was discontinued by Millendo

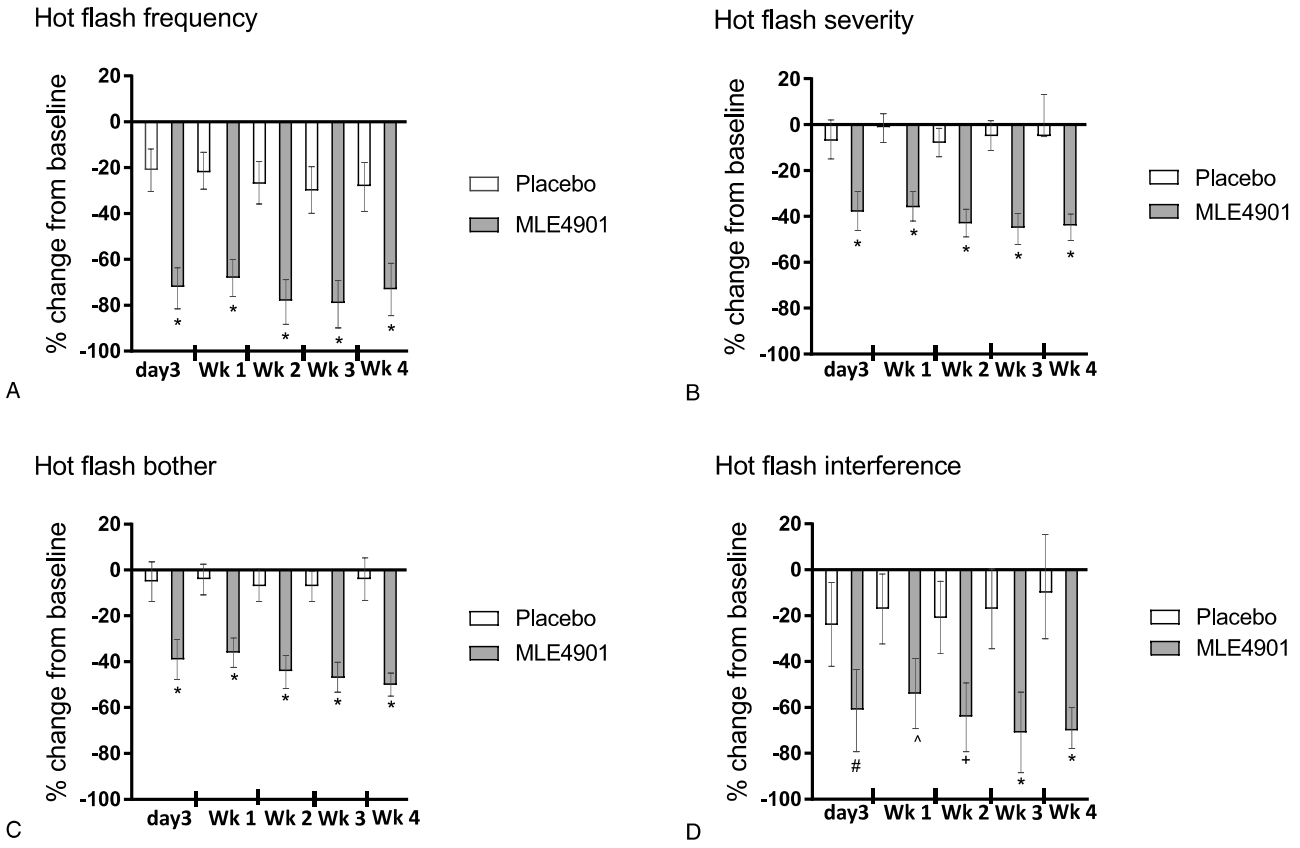

FIG. 2. Hot flash frequency (A), severity (B), bother (C), and interference (D) outcomes: results are presented as percentage change with 95% CIs from baseline at each time point during the treatment period (ie, on day 3 of treatment, and then weekly mean total for each week (wk) of the 4-week treatment period for both placebo (white) and MLE4901 (gray). Minimum $n = 33$; maximum $n = 37$. $^{*}P < 0.0001$, $^{\#}P = 0.0006$, $^{\wedge}P = 0.0011$, $^{+}P = 0.0001$. Week 4 data adapted from Prague et al, *Lancet*, 2017[18].

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Prague J., et al. Menopause. 2018 Aug;25(8):862-869.

Osanetant: Clinical Development Plan

- Acer is partnering with leading universities to design & conduct a clinical trial to evaluate osanetant in various patient populations with iVMS

- These include patients with medically or surgically iVMS (may include any/all of the following):
  - Women who are BRCA+ and have had a PBSO
  - Men with HR+ Prostate Cancer receiving leuprolide
  - Women with HR+ Breast Cancer receiving tamoxifen

- This initial Phase 1/2 trial would evaluate:
  - PK/PD and Safety, including physiologic PD
  - Identify the optimal dosing strategy to advance into further efficacy studies in minimizing the iVMS symptoms
  - Subject to additional capital

Osanetant: Exclusivity / Timelines

- Osanetant would be a New Chemical Entity (NCE) in the US, and as such would be eligible for five years' market exclusivity from potential FDA approval
- Additional exclusivity (e.g. Orphan Drug Designation) will depend upon indication(s) and development pathway chosen
- Anticipate IND submission in 2H 2020
- Aim to initiate Phase 1/2 trial by end of 2020, subject to additional capital

Financial Overview

- Cash
  - $12.1M as of December 31, 2019
  - Expected to have sufficient capital to fund current operations through end of 2020, excluding support for EDSIVO™ development and precommercial activities and planned osanetant clinical trial
- Capitalization as of December 31, 2019
  - 10.1M shares of common stock outstanding
  - 11.5M shares of common stock fully diluted
- $87M invested through August 2018 financing

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 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 induced Vasomotor Symptoms (iVMS) where Hormone Replacement Therapy (HRT) is likely contraindicated

- 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
  - Potential expedited paths for development through specific FDA-established programs

- Multiple anticipated key regulatory milestones:
  - ✓ EDSIVO™ FDRR[1] appeal denied but exploring possible paths forward: **Q1 2020**
  - ✓ ACER-001 (UCD) pivotal BE trial completion: **Q1 2020**
  - Osanetant IND submission: **2H 2020**
  - Osanetant Initiate Phase 1/2 PK/PD/safety trial[$]: **End 2020**
  - ACER-001 (UCD) NDA submission*[$]: **Q1 2021**

- Expected to have sufficient capital through end of 2020, excluding support for EDSIVO™ development and precommercial activities and planned osanetant clinical trial

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[1]Formal Dispute Resolution Request
[$]Subject to additional capital
*Assuming successful outcomes of additional nonclinical work and 12-month long-term stability data