UPDATE ON DRUG DEVELOPMENT

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Acer is working closely with key opinion leaders and patient advocacy groups to provide a compelling treatment option for patients with MSUD and urea cycle defects (UCD).

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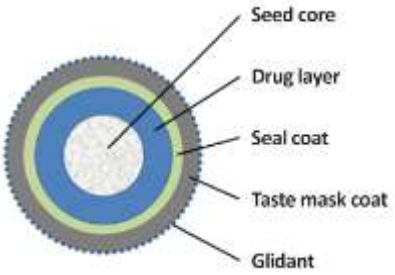
ACER-001 is a new formulation of phenylbutyrate being developed for MSUD that provides significant differentiation from other approved formulations of phenylbutyrate. It is a taste-masked, immediate release formulation of sodium phenylbutyrate, provided as an oral powder for reconstitution in liquid. Phenylbutyrate has been shown to reduce leucine levels in some individuals with MSUD, and may prove to be an effective adjunct to diet in the treatment of this disease. The goal of ACER-001 development is to improve palatability of sodium phenylbutyrate with a new formulation and show bioequivalence to the current drug, Buphenyl, in a clinical trial.

The product design is shown below.

Multi Layer Coated Particle:



Characteristics of current forms of phenylbutyrate compared with ACER-001:

Phenylbutyrate Formulations			
	ACER-001	RAVICTI	BUPHENYL
Efficacy/Safety in UCD	✓	✓	✓
Efficacy/Safety in MSUD	✓	X	✓
Palatability / Compliance	✓	✓	X
Relatively Reasonable Orphan Pricing	✓	X	✓

The first clinical trial of ACER-001 will be a study to show bioequivalence to Buphenyl, which is currently planned for 2019. The development plan for ACER- 001 has been delayed because manufacturing ACER- 001 has been difficult and has taken a lot of time to develop, resulting in this new timeline. Nevertheless, Acer intends to submit a new drug application (NDA) for UCD in end of Q4 2019 with anticipated sNDA submission for MSUD about a year later. Acer holds orphan drug designation in MSUD, and plans for advantageous orphan pricing with a robust program to support reimbursement and patient access.

The typical drug development/clinical trial process was briefly discussed at the MSUD Symposium, and is outlined below: