



EPI-743 Update BioElectron

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info@bioelectron.com

Agenda

Overview of Challenges

Development Status

Regulatory Process

Next Steps

Challenges

Disease heterogeneity

Absence of validated natural history

Absence of validated clinical trial endpoints

Patient logistics

Absence of clinical/regulatory precedent

Disease rarity

Disease severity

Development Status

18 clinical trials completed/ongoing

>400 patients treated

>1M clinical doses

Safety & tolerability

Predictable pharmacology

Clinical effect

New Drug Application Process: Leigh Syndrome

1. Non-clinical
2. Chemistry manufacturing and controls
3. Regulatory
4. Clinical

Next Steps

Regulatory meetings for EPI-743 approval: US, EU, & Japan

