



BioPharmaceuticals, Inc.

# **Galanin Gene Delivery for Temporal Lobe Epilepsy**

Disruptive Technology  
targeting an unmet medical need

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# Our Approach to Treating Temporal Lobe Epilepsy

- One-time delivery of a seizure inhibiting gene only to the site of epileptogenesis
- Localized secretion of the anti-convulsant neuropeptide galanin to avoid ectopic action on other parts of the brain
- Stable, very long term effect anticipated (10 years or more)

# Why is a new Approach Necessary?

- ~ 1/3 of patients with epilepsy do not have good seizure control
- New AEs have not significantly impacted the medically refractory population
- Only a subgroup of patients are able to obtain reasonable resolution after brain tissue removal

# What are the Limitations of other Approaches?

- New AEs have not significantly resolved intractable epilepsy
- Significant side effects.
- Neurosurgical resection is only possible if the brain tissue is not critical matter, and still results in trauma and side effects associated with loss of unique brain matter.

# Our Approach

- Combine the best of Neurophysiology, Neurosurgery & Gene Therapy
- A gene delivery system that is safe and well tolerated, mediating long term therapeutic protein production at the site of injection.
- Integration with a technology provides all target cells the ability to make the therapeutic product

# Our Progress & Plans

- Efficacious & safe in small animal models with correction of seizure phenotypes (SBIR)
- Collaborative relationship with Medtronic for support with preclinical development and with device and administrative support
- Completion of ongoing large animal studies will finalize clinical protocol, support IND submission (ETP)



# The Future

*The NEW ENGLAND JOURNAL of MEDICINE*

ORIGINAL ARTICLE

## Adenovirus-Associated Virus Vector– Mediated Gene Transfer in Hemophilia B

Amit C. Nathwani, M.B., Ch.B., Ph.D., Edward G.D. Tuddenham, M.B., B.S., M.D.,

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EDITORIAL



## Merry Christmas for Patients with Hemophilia B

Katherine P. Ponder, M.D.