ENABLING NOVEL TREATMENTS FOR NERVOUS SYSTEM DISORDERS BY IMPROVING METHODS FOR TRAVERSING THE BLOOD-BRAIN BARRIER:

A WORKSHOP

Session 2

Traversing the BBB—Pre-Clinical to Clinical Translation

Co-Moderators:

E. Antonio Chiocca, Harvard Medical School
Danica Stanimirovic, National Research Council of Canada

Session I: Traversing the BBB –
Pre-clinical to Clinical Translation

Session Overview

DANICA STANIMIROVIC, National Research Council of Canada (CO-MODERATOR)

E. ANTONIO CHIOCCA, Harvard Medical School (CO-MODERATOR)

Presentations

BALU CHAKRAVARTHY, National Research Council of Canada

ALEXANDRA GOLBY, Brigham and Women's Hospital

RUBEN BOADO, Armagen

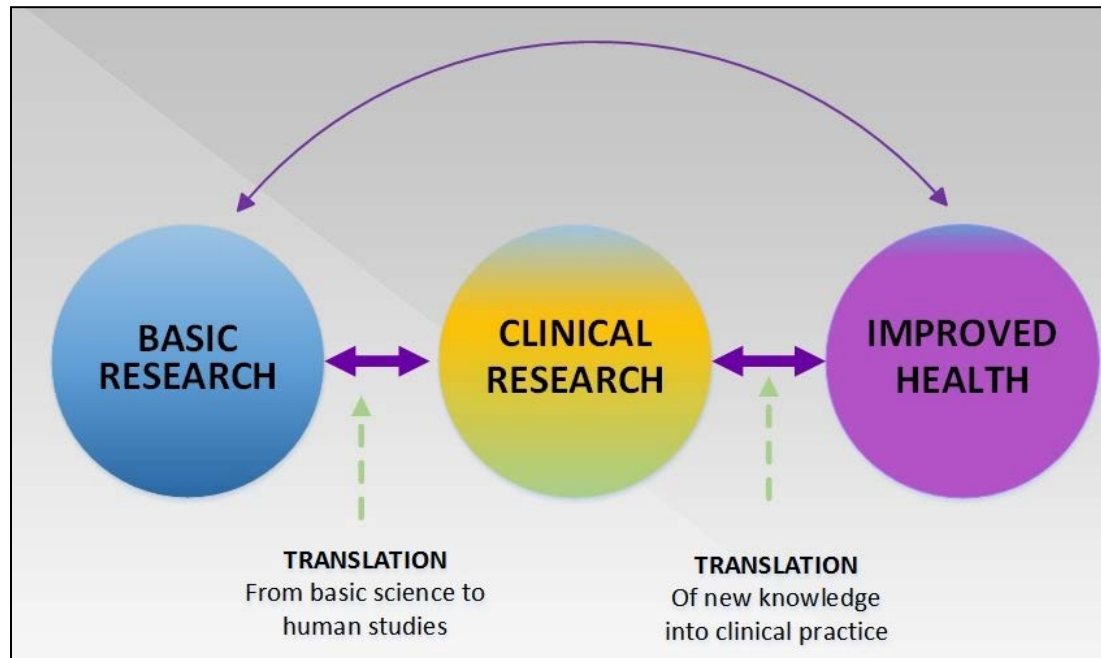DAHAVALKUMAR SHAH, State University of New York at Buffalo

Discussion

Session I: Traversing the BBB – Pre-clinical to Clinical Translation

Session Objectives:

- Discuss the translation – from late preclinical work to clinical trials – of delivery strategies for traversing the BBB, including delivery of synthetic molecules, biologics, and gene therapy.
- Describe the limitations of current methods for traversing the BBB and identify research and other potential next steps that would move the field forward.

Challenges of translation in CNS/BBB

- Relevance of **animal models**

- **Small brain vs. big brain**

- **Species** differences

- Planning **pre-clinical studies** to 'mimic' clinical studies (outcome measures)

- **Biomarkers** of target engagement (and surrogates)

- **Toxicity, immunogenicity**

- **PKPD** – Scale up across species, physiological modeling