



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

Panel 1

**Regulatory Considerations in Development of Methods
for Traversing the BBB**

Moderator

**Francesca Bosetti, National Institute of Neurological
Disorders and Stroke**

Panel I: Regulatory Considerations in Development of Methods for Traversing the BBB

Session Overview

FRANCESCA BOSETTI, National Institute of Neurological Disorders and Stroke,
(MODERATOR)

Panel Remarks

DOUGLAS HUNT, Armagen

VIKRAM PATEL, Food and Drug Administration

Discussion



Panel I: Regulatory Considerations in Development of Methods for Traversing the BBB

Session Objectives:

- Discuss approaches, tools, and lessons learned from other regulatory domains that may advance the development and translation of novel methods to traverse the BBB.
- Identify specific barriers and opportunities in the regulatory domain related to the development and application of methods for traversing the BBB.
- Explore issues related to critical attributes and potency assays; safety, including immunogenicity and CNS toxicity; and animal models, including appropriate species selection.
- Explore best practices and strategies to facilitate regulatory consideration of novel technologies for traversing the BBB.



- Best practices in preclinical studies (using small/large animal models) to improve validity and accelerate clinical evaluation: regulatory steps and efficacy/safety requirements.
- Potential use of imaging agents and biomarkers in support of clinical efficacy and other surrogates to support/ accelerate approval.
- Regulatory challenges for developing drug conjugates enabled to traverse BBB, as well as for excipients or agents that enable drugs traverse BBB, and for new mechanical approaches (e.g., focused ultrasound disruption).
- Phase 0 trials to assess BBB penetrations and new analytical methodologies.

