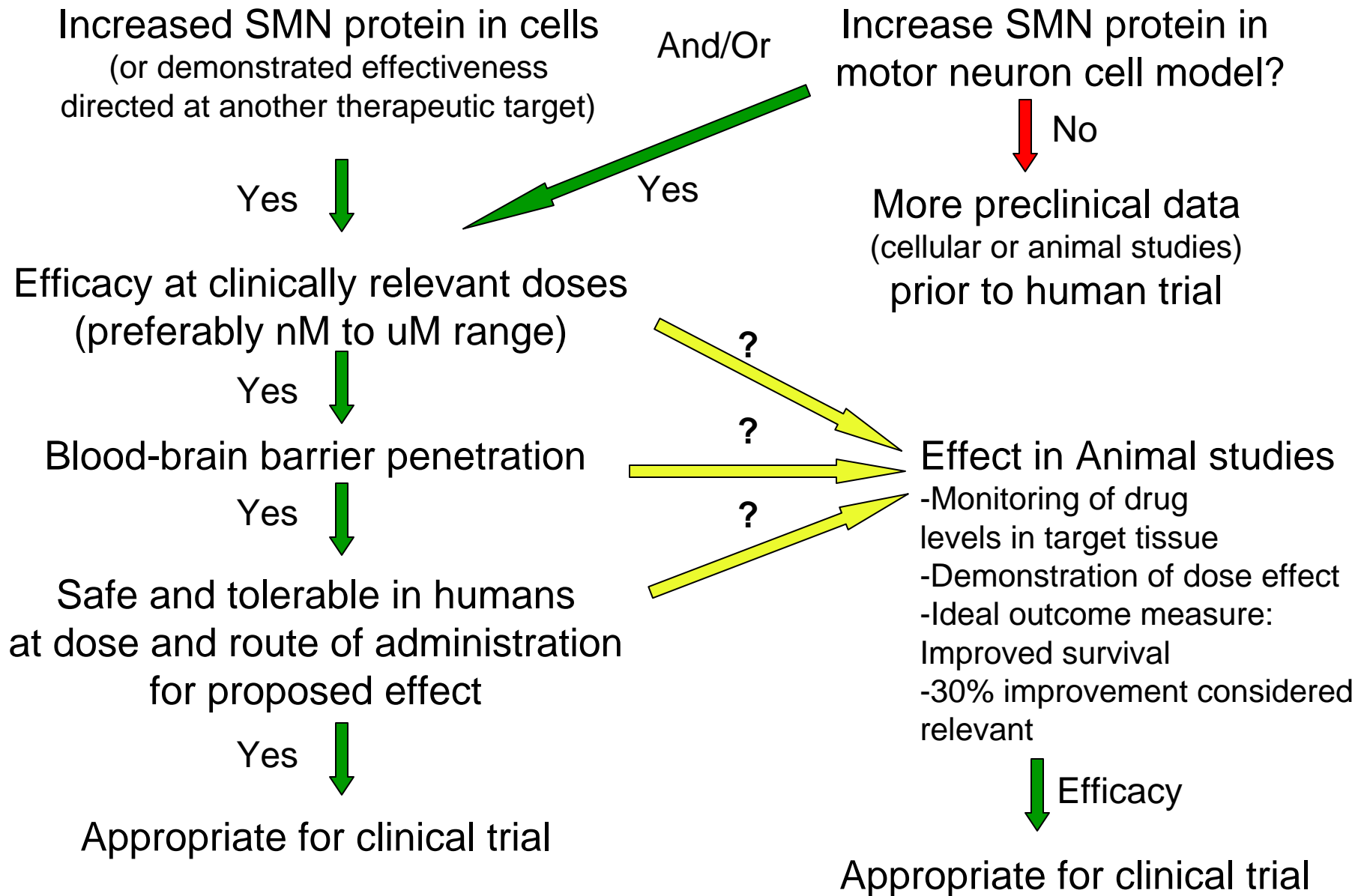


SMA Summit on Drug Development

Session 8

Evaluating Pre-clinical and Clinical
Compounds





<http://www.smaproject.org/TAP-SMA.html>

Evaluating Pre-clinical compounds: models, efficacy and safety

- What evidence in SMA models is needed?
- How should negative data in SMA models be used in decision making?
- What is the role of non-SMN targeted mechanisms of drug action?
- What pre-clinical safety studies are necessary for infants and children?
- What factors influence the SMA market?