

Experiences with Submitting a Pediatric Probiotics IND Application to the FDA

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The Beginning

- During fellowship, sought to initiate clinical trial of probiotics to prevent recurrent UTI in children
- Applied to and awarded a Thrasher Foundation grant



- Played by the rules on Investigational New Drug (IND) issue: “diagnosis, cure, mitigation, treatment, or prevention of disease in man”



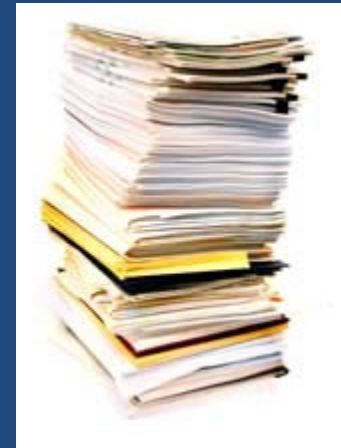
FDA Ruling on Need for IND

- Center for Biologics Evaluation and Research (CBER), Office of Vaccine Research and Review (OVRR) ruled that IND was needed



Information Gathered for IND

- Drug Master File (chemistry, manufacturing, and controls data)
- Safety data from ~20 adult trials and ~10 pediatric trials (one trial with HIV patients, all trials tested non-UTI indications), none performed in U.S.
- *In vitro* data
- Trial design



IND Ruling by the FDA

- Just within 30 day limit
- Clinical hold pending a number of requests for additional CMC-related information, as well as requirement to conduct an **adult safety trial in women prone to recurrent UTI**
- Due to FDA request for adult trial being outside of scope of Thrasher Foundation, funding not granted, IND withdrawn



Conclusions

- If 30 clinical trials is insufficient safety data, now what?!?
- With this regulatory climate, how will any novel treatments be approved for children with urologic disorders?
- FDA, Office of Dietary Supplements (ODS), and the National Center for Complementary and Alternative Medicine (NCCAM) are co-sponsoring a systematic review of probiotic safety, Safety of Probiotics Used to Reduce Risk and Prevent or Treat Disease.

