

Product/Topic: Gaucher Disease- a collaborative approach from EMA and FDA

IND/NDA Number: multiple

PIP number: multiple

Indication: Gaucher disease

List of Issues Discussed

Multi-arm, multi-company trials
Extrapolation
Primary Endpoint
Trial duration
Nonclinical aspects

Comments That Were Shared

FDA and EMA recognized the need for multi-arm, multi-company trials based on feasibility concerns and challenges, A reduction in the total number of children to be included would be achieved, compared to separate controlled trials, as the same control arm would serve more than one product.

FDA and EMA agreed that extrapolation of efficacy from adults to children can be considered applicable to visceral and hematologic endpoints within Type 1 Gaucher disease.

FDA and EMA agreed that a change in normalized hemoglobin level that demonstrated non-inferiority to treatment with an active control (imiglucerase) should be the primary endpoint. It is scientifically robust and it has been used in prior approvals.

There was agreement that the trials should be 2 years in duration for the primary endpoint with extensions of at least 3 years but with recommendation for 5 years to collect long-term efficacy and safety data (e.g. effects on bone, growth, puberty and pulmonary function).

FDA added and EMA agreed that the need for juvenile animal toxicity studies should be decided on a case-by-case basis.

Note: FDA's and EMA's comments are preliminary and not binding and they do NOT constitute regulatory action.