

Follow-On Biologics - Evidence to Assure Patient Safety

A Conceptual Approach to Crafting Legislative Language Regarding Patient Safety
(an elaboration of comments made Dennis Cotter atCMPI Capitol Hill July 15th Capitol Briefing)

I. - Relative Stringency FDA Patient Safety Requirements for Rare Diseases

(e.g., RCT sample size, trial duration, number and type of endpoints)*

Size of Patient Population	Phase III	Phase IV
Relatively Small	lower	higher
Relatively Large	higher	lower

* justification for distinction: theories advanced under compassionate use, life-support/life-sustaining, end-of-life

@ perhaps use Orphan Product definition

if using Orphan Product definition distinguish between small vs. large patient numbers categorization could be determine statistically by known epidemiology such as CDC, NORD, etc.

II. - Relative Stringency FDA Patient Safety Requirements for Biological Treatment of Non-Rare Acute & Chronic Diseases

(e.g., RCT sample size, trial duration, number and type of endpoints)

Size of Patient Population	A. Using a 'hard' clinical endpoint		B. Using a surrogate (lab value) endpoint	
	Phase III	Phase IV	Phase III	Phase IV
Relatively Small	lower	higher	higher	higher
Relatively Large	higher	lower	higher	higher

notes:

Phase III - FDA pivotal clinical trial requirements

Phase IV - FDA post-market surveillance requirements

FDA requirements such as Good Manufacturing Practices are not addressed

a case study that addresses implications of not publishing final results from a Phase IV study regarding erythropoietin (epoetin) can be found at www.mtpqi.org

source: MTPPI