# Clinical Trial Development: One Sponsor's Perspective

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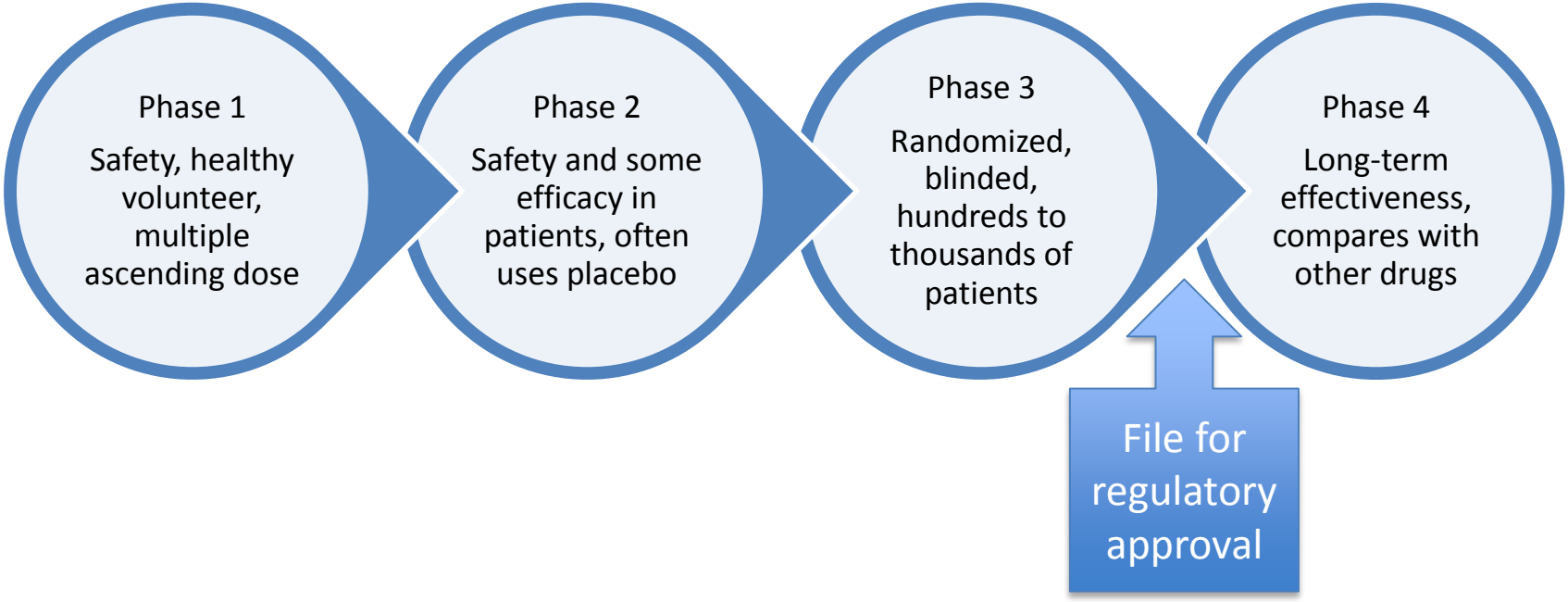
- The forward-looking statements in this presentation speak only as of the original date of this presentation, and we undertake no obligation to update or revise any of these statements.

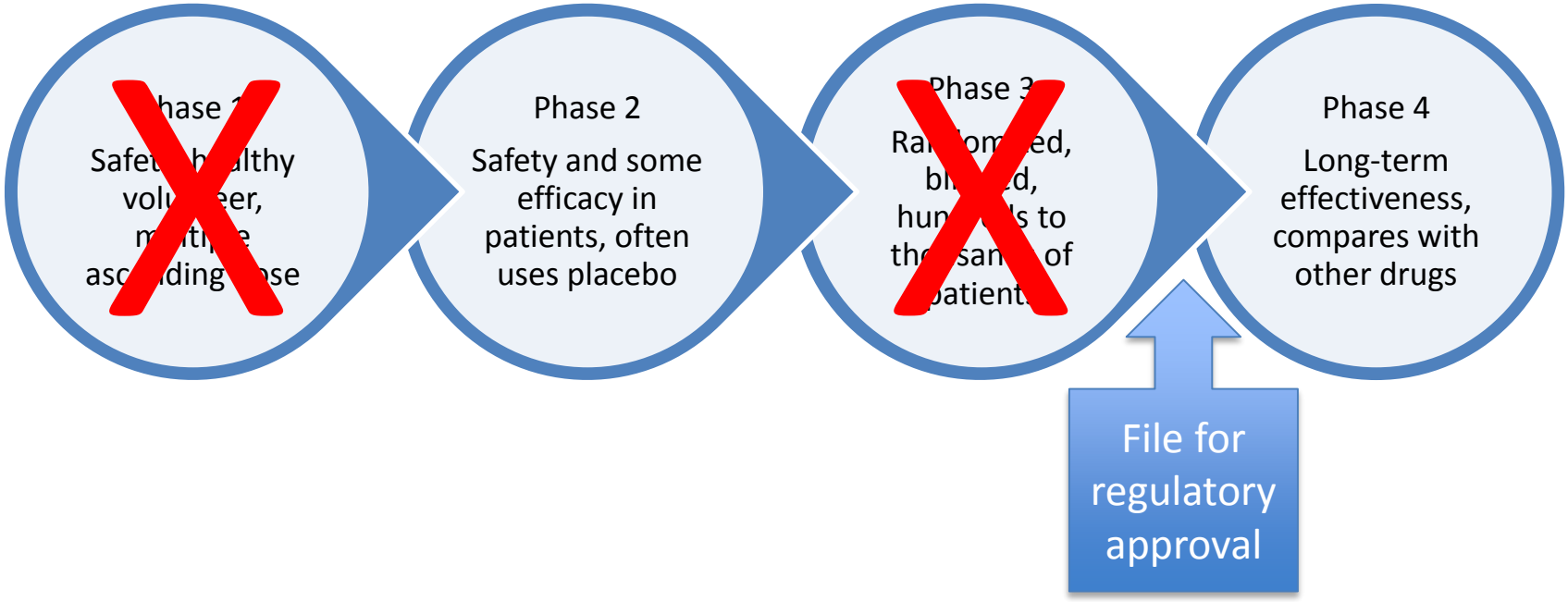
# What goes into clinical trial development?

- Typical drug development



# What goes into clinical development in rare diseases like DMD?

- Faster with fewer subjects and less data



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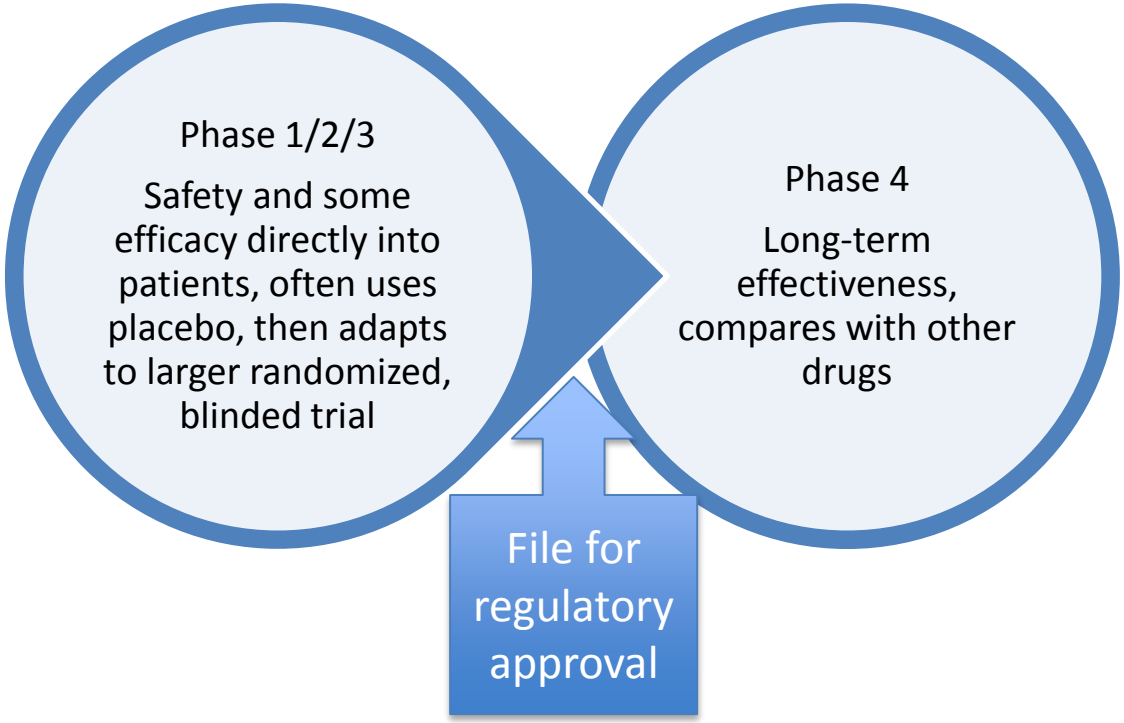
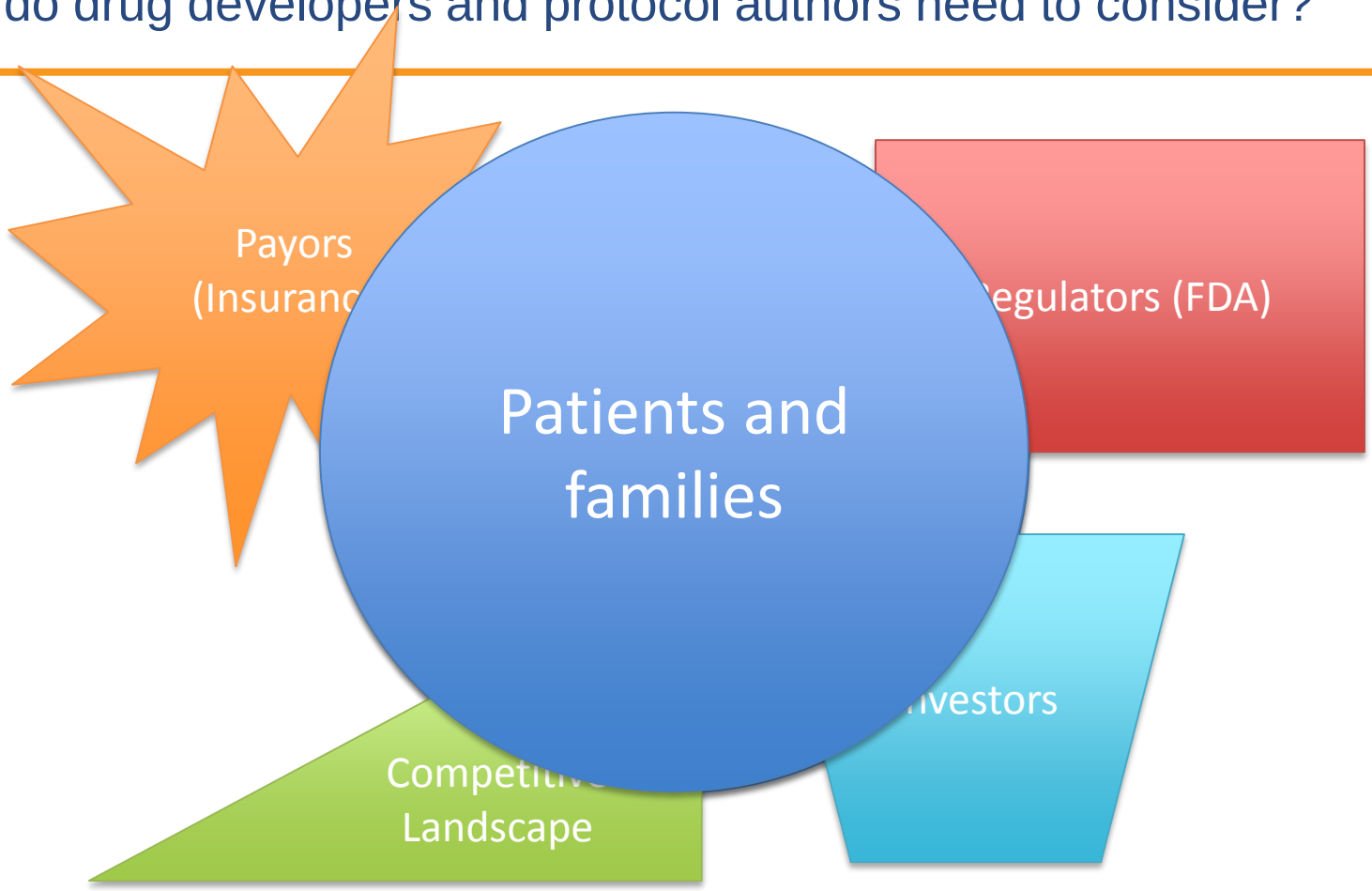


# Whom do drug developers and protocol authors need to consider?



# Potential impact of clinical trials on participating families

- Fatigue
- Guilt
- 2nd guessing
- Frustration
- Isolation

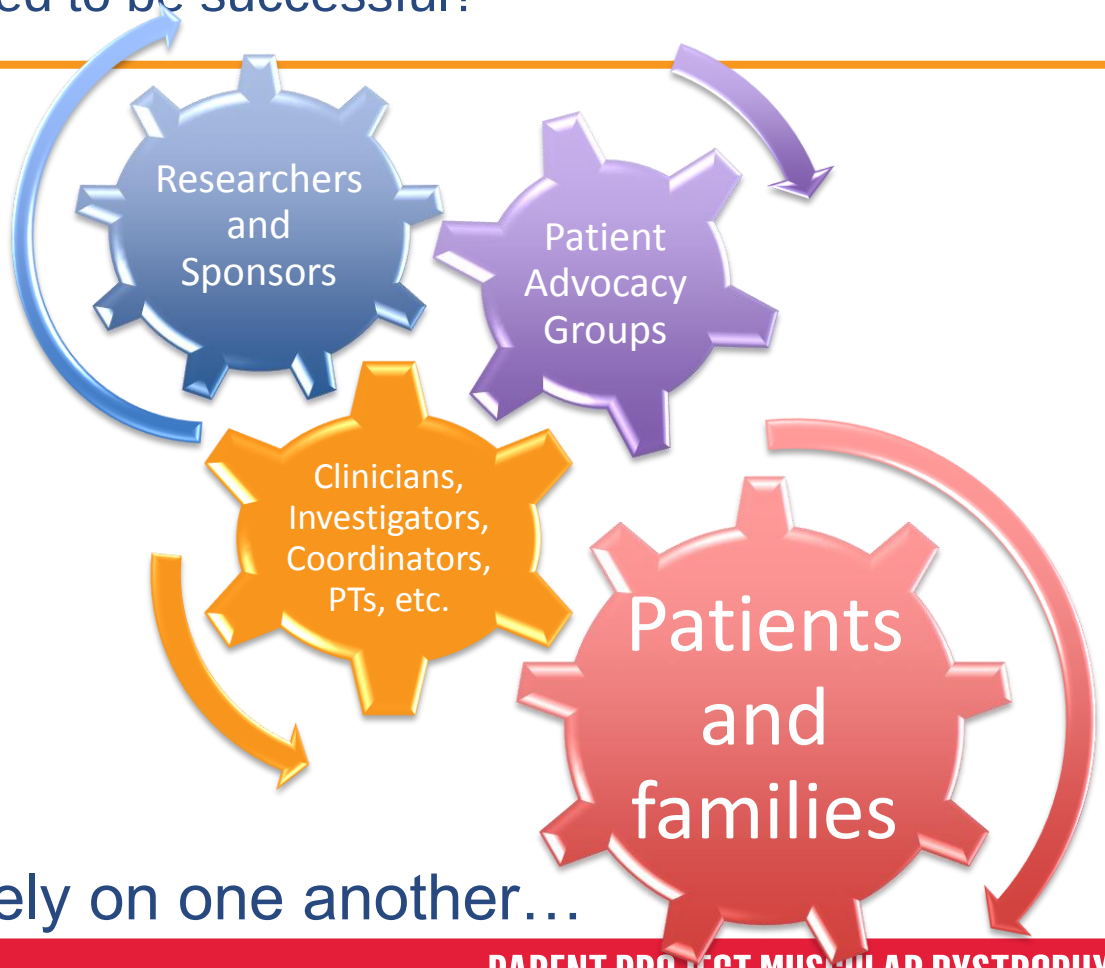
*Design features influenced by patient advocacy organizations:*

- Informed consent process
- Participant selection
- Travel support
- Steroid management
- Biopsy collection, handling, and follow-up care
- Activity monitoring
- External data monitoring committee membership
- Communications
- Data sharing
- Psychosocial burden

# Participating families impact on clinical trials

- Benefit of advancing the science, and improving understanding of:
  - Disease and its burdens
  - Investigational drug safety and efficacy
- Possible *risks* of sharing any perceived therapeutic benefit with broader community during clinical trial:
  - Creation of false hopes and expectations
    - Early phase may not be designed (e.g., have enough participants) to show clinical benefit;
  - Confusion by other participating families who may not see same results
    - Possibility results may fluctuate and/or be inconsistent across participants;
  - Disappointment by families who cannot participate in the same trial
  - Influence on broader community
    - Even drug developers, who may inadvertently become bias in their interpretation of risk/benefit for a potential therapy.

# What is required to be successful?




We need to rely on one another…

# TO ALL FAMILIES, ADVOCACY, INVESTIGATIVE SITES, AND RESEARCHERS…