N1C 2024 Annual Meeting

Getting to Clinic

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Disclosures

I have consulted for Biomarin, Neurogene, Taysha, Zogenix, Marinus, Ultragenyx, Encoded, and Capsida.

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I also serve on the advisory board for the non-profit foundations SLC6A1 Connect, Project 8P, Ring14 USA, FamilieSCN2A and N=1 Collaborative.

What does it take to get an n of 1 into clinic?

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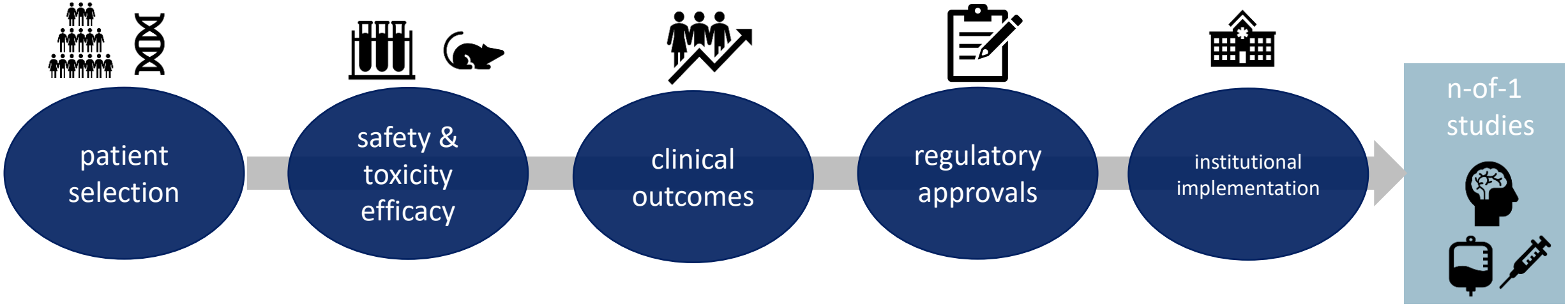
Implementing individualized genetic medicines



Regulatory Submissions

Everything you know and your best justification:
- Preclinical data justifying prospect of benefit and safety profile
- Identified outcome measures
- Safety and efficacy monitoring plan
- Dose justification
- etc.

Institutional Implementation

Considerations:
- Study Team
- Trial start-up and maintenance
- Funding
- Institutional buy-in
- Continuation and discontinuation criteria

Study Team

- PI
- Coordinator
- Nursing
- Regulatory support
- Multi-disciplinary care team
- Database management
- Compliance
- Pharmacy
- Procedure center
- Consent monitor
- DSMB

Start-up

- Two phase approach
    - While drug development and optimization is occurring, you collect rigorous baseline data (master protocol ?)
    - Treatment phase
- Recommend feasibility meetings and mock visits
- Standard start up processes

Funding

- Survey: Historic and currently funded trials
- Distributed to N=1 Collaborative members
- 20 valid responses (United States, Germany, the Netherlands)

Funding

- You have to get creative in current state, this is not ideal and leads to inequity

- Insurance: research compliance teams can evaluate what would be considered standard of care and therefore billable to insurance
    - Just because it is billable doesn't mean it will be covered

- Non-standard of care cost need to be covered by another source along with research cost

Institutional Buy-in

- This is the hardest component if never done before

- Find an Executive Champion

- Be willing to repeat yourself, compare to standard trials, show people why this is not crazy

Continuation – Discontinuation Criteria

- This is different than a traditional trial

- Discuss with patient / family what success looks like and have some milestones you can anchor to later

- Clearly outline the decision process and owners up front

- Criteria may be different in different phases

Treat the Patient

Super Exciting!!!!

Treat the Patient

Some healthy anxiety is a good thing

Treat the Patient

Focus on safety!

Resources

https://www.n1collaborative.org/resource-navigator