



FA Drug Development

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Disclaimers

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NAF and Reata encourage all attendees to consult with their healthcare provider for medical advice.

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Friedreich's Ataxia: Ultra-Rare, Progressive, Neuromuscular Disease



- **Ultra-rare genetic disease**

An estimated 5,000 patients are diagnosed in the U.S.⁵



- **Relentlessly progressive loss of motor function**

- Typically diagnosed in teens⁶, requires mobility aids in twenties⁷, mean survival is mid-thirties⁸



- **No approved therapies before now**

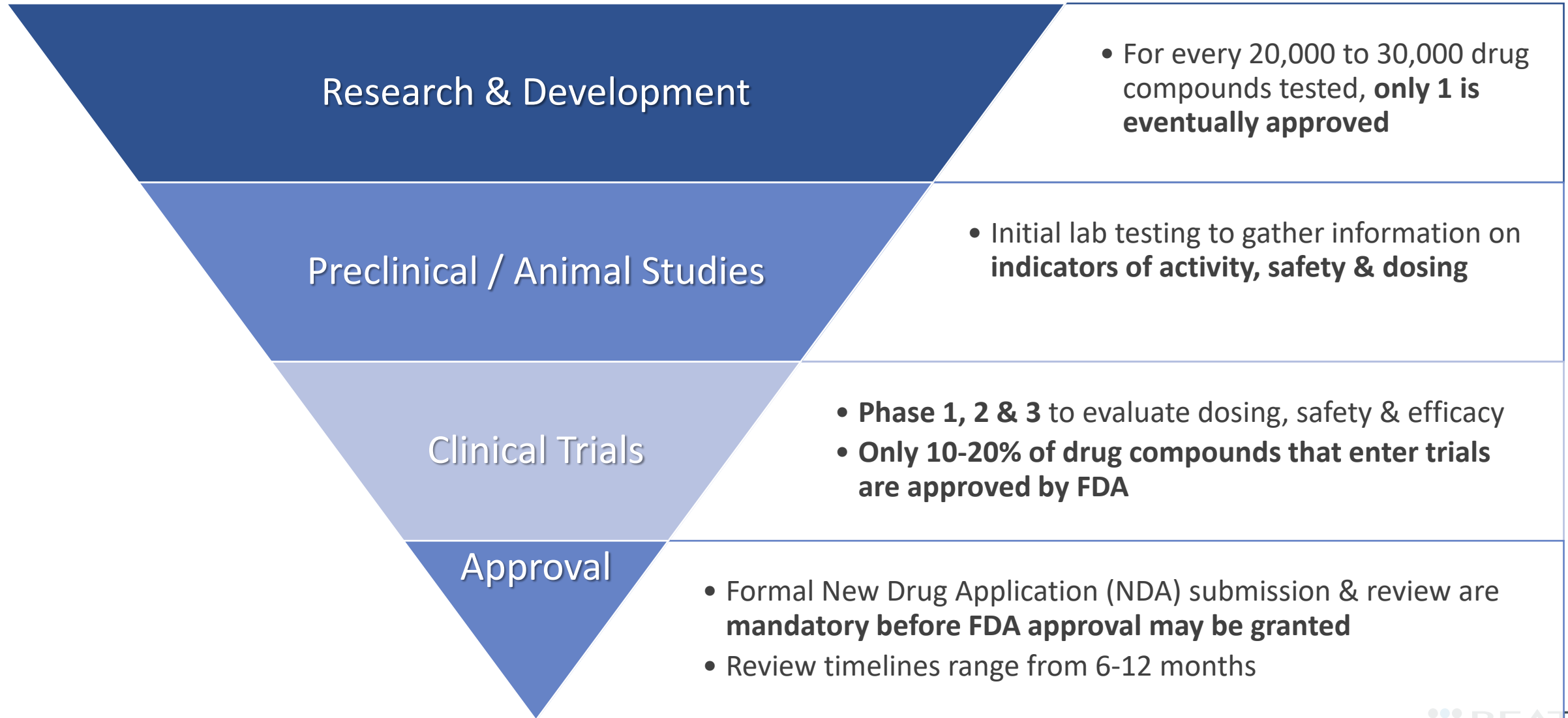
¹Cook A., Br Med Bull. 2017; ²Poburski D., Biology Open 2016; ³D'Oria V., Int. J. Mol. Sci. 2013; ⁴Paupe V., PLoS ONE 2009; ⁵U.S. claims data and projected diagnosed, ⁶Rummey C., Neurol Genet 2019; ⁷Rummey C., E Clinical Medicine. 2020; ⁸Tsou A.Y., J Neurol Sci 2011

A detailed microscopic image of a neuron, showing its cell body (soma) and several branching processes (dendrites and an axon) extending outwards. The neuron is stained in shades of blue and green, set against a dark background.

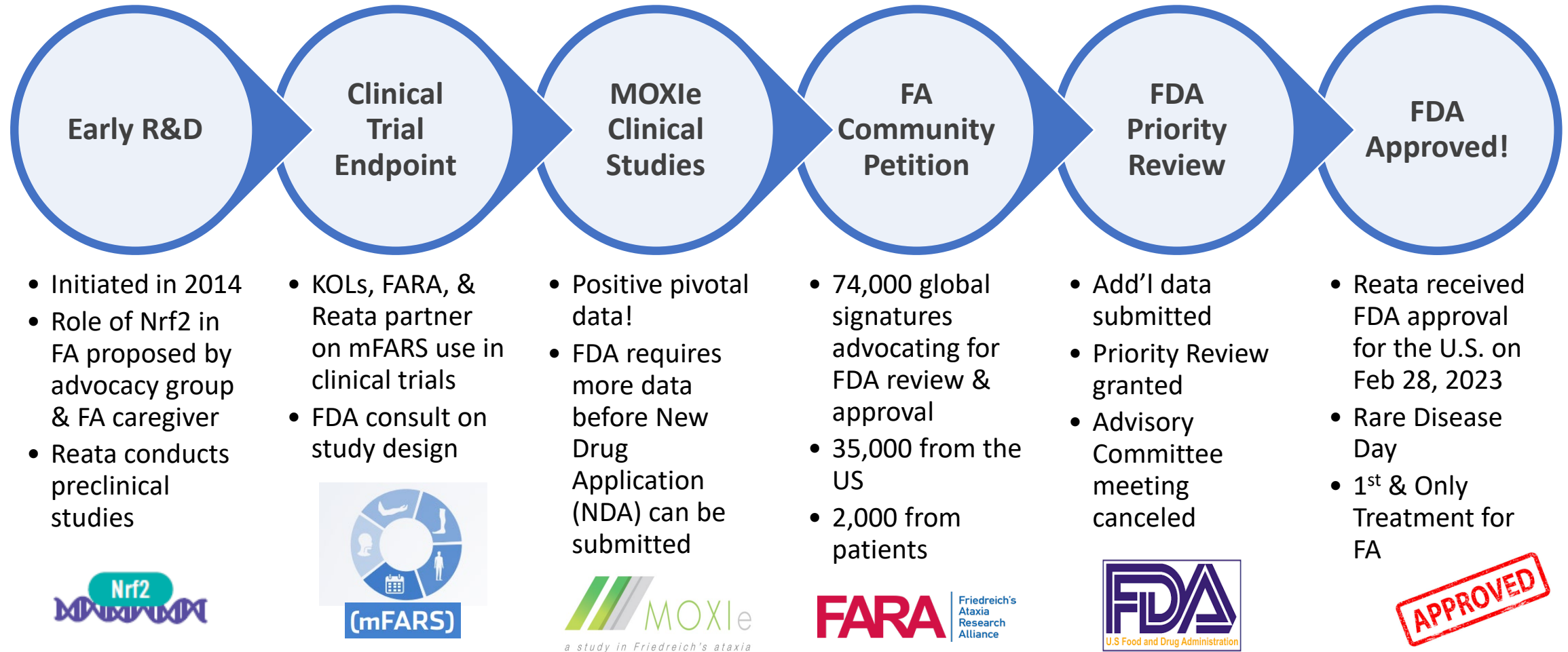
FA Community has Worked Diligently to Facilitate Drug Development

- **Better understand mechanism and course of disease**
- **Support research & development through grants and funding**
- **Develop clinically meaningful endpoints**
- **Design robust, viable clinical trials**
- **Consult with FDA on regulatory path**

Developing a New Drug May Take ~12-15 Years



Reata's Journey From Bench to FDA Approval





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NOW APPROVED: SKYCLARYS is a prescription medicine for the treatment of **Friedreich's ataxia** in adults and adolescents aged 16 years and older.

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Two opportunities to watch:
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Presented by Matthew Lafleur

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Please see back for Important Safety Information.



Patients' **ROLE** in drug development: Be a part of research!



We thank FA patients, their caregivers, advocacy & all

