UNITED MITOCHONDRIAL DISEASE FOUNDATION®

Leveraging Patient-Derived Natural History Data When Designing Clinical Trials

FDA Workshop
Developing Therapies for Primary Mitochondrial Diseases: Bridging the Gaps

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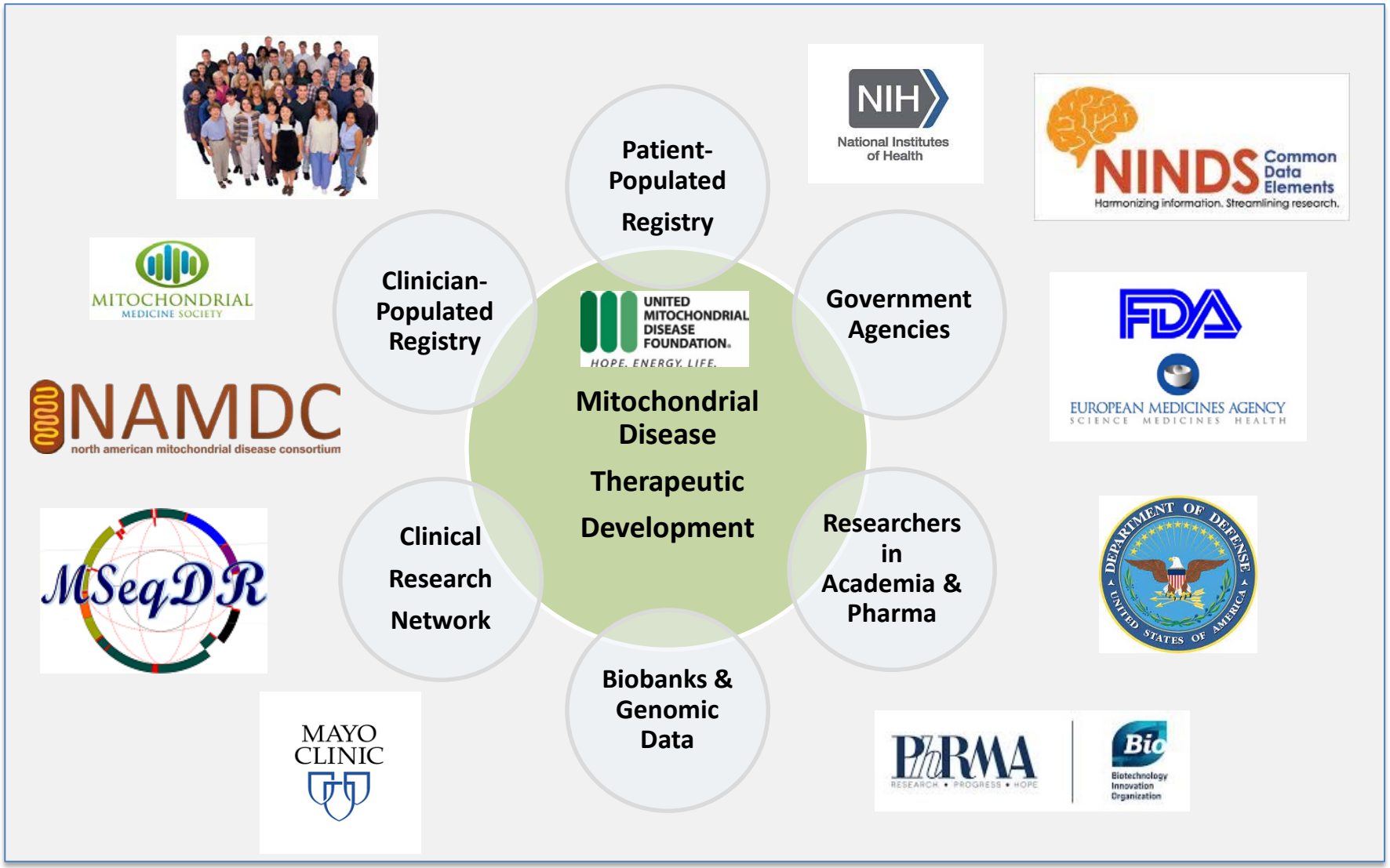
UMDF

Mitochondrial Disease Ecosystem



Mitochondrial Disease Community Registry

www.umdf.org/registry

Who?

Patients, caregivers & family members

Confirmed diagnosis ***NOT*** required

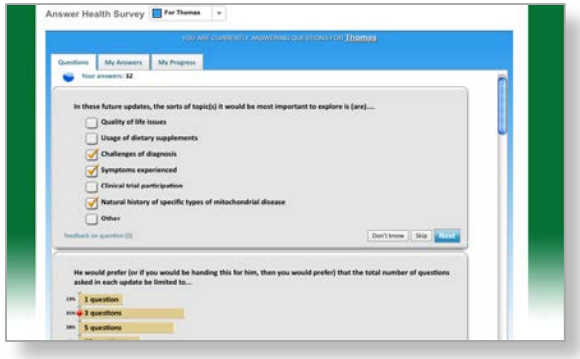
Why?

Need patient data collected over time in order to improve diagnoses and develop treatments

Key

Registrants have full control of privacy settings

- Allow, deny or “ask me”
- Who can see anonymous data
- Who can analyze anonymous data
- Who can contact you about research studies and clinical trials



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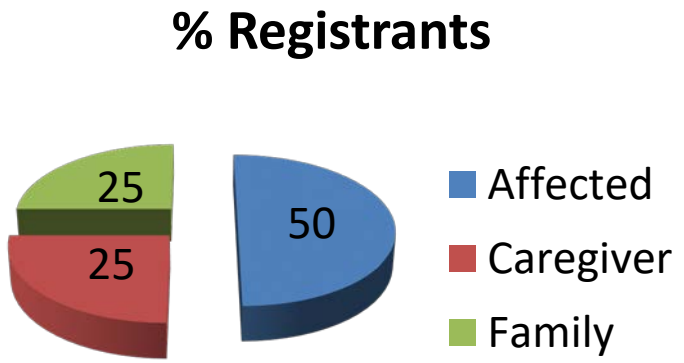
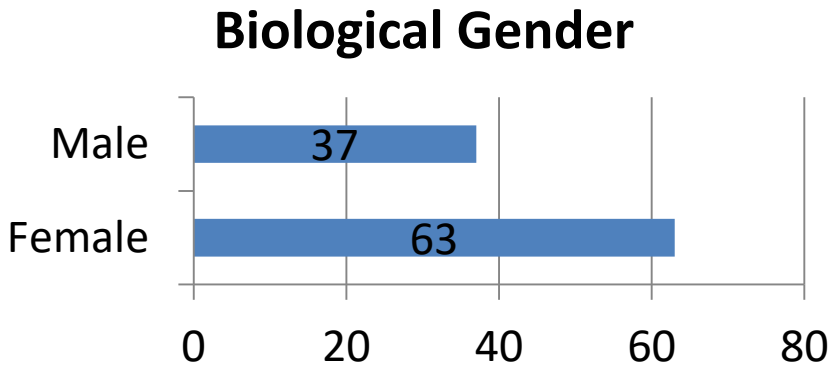
PLEASE REGISTER & SHARE!

MDCR Current Status

- June 2019: ~2,500 registrants
- Single static survey
  - ~100 Q's/~50K Responses
  - Baseline demographics
  - Diagnostic State
  - Opinions on patient-centered drug development
- International: 46 countries represented!
  - ~90% from the US





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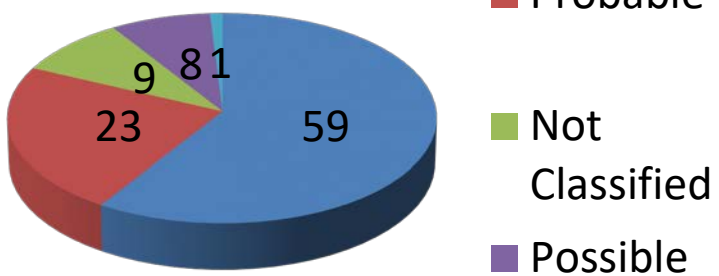
MDCR Data Snapshots

Self-Reported Diagnostic State



Sample of Mitochondrial Disease Types Currently Represented in MDCR

Mitochondrial DNA Mutation Syndromes
LHON, LHON+, MELAS, MERRF, MIDD, MILS, NARP

Mitochondrial DNA Deletion Syndromes
CPEO, KSS, ADOA

Nuclear Gene Disorders
Leigh's, MNGIE, Alper's, SANDO

Biochemical Disorders
Complex I, Complex II, Complex III, Complex IV, Complex V, CoQ Deficiency

System-Based Disorders
Encephalomyopathy, Encephalopathy, Mitochondrial Myopathy

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Near-Term Growth Initiatives

- Inter-connectivity
  - Ensuring MDCR can connect/share with other data repositories
- Data Collection Diversification
  - Longitudinal Patient-Reported Outcomes
  - Patient Genetic Data
  - Patient Biosamples & Virtual Repository
- Globalization
  - Establishing a network platform with international PAG partners
    - Ensure we are identifying and characterizing as many patients as possible to improve readiness for clinical trials

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