

Our Next Steps

Addressing Challenges and Improving Outcomes for Wolfram Syndrome Patients

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Action Items in 2009



1. Patient registry
2. Longitudinal study
3. Biomarkers
4. Service clinic (multi-disciplinary)
5. Clinical guidelines
6. Animal and Cell models
7. Drug targets/Drug development
8. International Collaboration
9. Share data and reagents
10. Cure



Objectives

1. Improving Patient Outcomes:

Develop clinical guidelines for early diagnosis, treatment, and QOL improvement. Explore innovative therapies, promote multidisciplinary care, and offer psychological support.

2. Engaging the Medical Community:

Raise awareness through conferences, workshops, and webinars. Encourage research funding, collaboration between clinicians, researchers, industry partners and advocacy groups.

3. Advocating for Public Policy Changes:

Push for better access to care, research funding, and support for affected families. Work with policymakers and advocacy groups to implement insurance coverage, centers of excellence, and early screening programs.

1. Develop Clinical Guidelines for Wolfram Syndrome Treatment

- Adapt existing format for rare diseases and finalize guidelines
- Publish a downloadable version on patient organizations' websites
- Incorporate a gene panel-based screening strategy
- Collaborate with patient organizations to disseminate guidelines to targeted specialists

2. Compile a List of International Clinical Trial Sites for Wolfram Syndrome

- Identify motivated and experienced physicians to conduct trials
- Ensure a diverse and representative selection of trial sites

3. Explore Novel Biomarkers for Wolfram Syndrome

- Conduct research to identify new biomarkers related to disease progression
- Implement blood draw protocols at multiple centers every six months to monitor biomarker levels

4. Connect Patient Registries

- Develop a plan to connect existing patient registries for Wolfram syndrome.
- Collaborate with patient organizations to gather and share patient data.

5. Create an Innovative Clinical Trial Protocol for Novel Therapies, including Gene Therapy

- Develop a clinical trial protocol for novel therapies, including gene therapy.
- Identify potential partners for collaboration on the clinical trial.