### FRIEDREICH’S ATAXIA TREATMENT PIPELINE

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<tr>
<th>Discovery</th>
<th>Pre-Clinical Development</th>
<th>IND Filed</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
<th>NDA Filed</th>
<th>Available to Patients</th>
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<tr>
<td>Modulation of Frataxin</td>
<td>Frataxin Replacement</td>
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<td>Frataxin replacement CTI-1601</td>
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<td>Retinoids</td>
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<td>Improve Mitochondrial Function &amp;</td>
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<td>Reduce Oxidative Stress</td>
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**Current Treatments**

- **Omaveloxolone (RTA-408) - Nrf2 Activator**
  - Jupiter Therapeutics & Murdoch Children’s Research Institute, Australia

- **EPI-743**
  - BioElectron

- **RT001 (dPufas)**
  - Retrotepe

- **(+ Epicatechin)**
  - Cardero Therapeutics and Mayo Clinic

- **Methylprednisolone**
  - Children’s Hospital of Philadelphia

- **TAK-831**
  - Takeda

- **MIN-102**
  - Minoryx Therapeutics

- **Dimethyl fumarate**
  - Ixchel Pharma & University Federico II, Naples, Italy

**Future Treatments**

- **CTI-1601**
  - Chondrial Therapeutics

- **EPO-mimetics**
  - STATegics

- **Ubiquitin Competitors**
  - Fratagene Therapeutics

- **BMN-290**
  - BioMarin

- **Resveratrol**
  - Jupiter Therapeutics & Murdoch Children’s Research Institute, Australia

- **Nicotinamide**
  - E-rare & German Research Foundation

- **RNA-based approach**
  - University of Texas Southwestern, Univ of Massachusetts

- **AAV-based approaches**
  - Adverum, Bamboo/Pfizer, PTC Therapeutics, Voyager/Neurocrine, IGBMC (Strasbourg, France), University of Florida

- **Cytokine mediated therapy / GCSF**
  - University of Bristol

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