

# Approved drugs for Mitochondrial disease

April, 2018

Drug		Type of disease	Country	Company
Raxone	<a href="http://www.santhera.com/">http://www.santhera.com/</a>	LHON	EU (under exceptional circumstances)	Santhera Pharmaceuticals

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# Clinical research, clinical study, and clinical trial in Japan

April, 2018

Compound		Type of disease	Status	Sponsor
Vatiquinone (EPI-743) (UMIN000010783)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/ctr.cgi?function=history&amp;action=list&amp;type=summary&amp;recptno=R000012615&amp;language=J">https://upload.umin.ac.jp/cgi-bin/ctr/ctr.cgi?function=history&amp;action=list&amp;type=summary&amp;recptno=R000012615&amp;language=J</a>	MELAS	Clinical study Phase 1	National Center Hospital, National Center of Neurology and Psychiatry
Vatiquinone (EPI-743) (JapicCTI-132349) (JapicCTI-142628)	<a href="http://www.clinicaltrials.jp/user/cteDetail.jsp?clinicalTrialId=12520">http://www.clinicaltrials.jp/user/cteDetail.jsp?clinicalTrialId=12520</a>	Leigh Syndrome	Clinical trial Phase 3	Dainippon Sumitomo Pharma Co., Ltd
Taurine (UMIN000029376)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000033379">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000033379</a>	MELAS	Clinical study Phase 3 (Application preparation)	Kawasaki Medical School
Taurine (UMIN000009358)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000010995">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000010995</a>	MERRF	Clinical study Phase 2	Kawasaki Medical School
Bezafibrate (UMIN000017984)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000020843">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000020843</a>	Mitochondrial Fatty Acid Beta-Oxidation Disorder	Clinical study Phase 2/3	Shimane University Hospital
Sodium pyruvate (UMIN000025880)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000029753">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000029753</a>	Leigh Syndrome	Clinical study Phase 3	Kurume University School of Medicine
Sodium pyruvate (UMIN000025874)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000029752">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000029752</a>	MELAS/MELA	Clinical study Phase 3	Kurume University School of Medicine
Sodium pyruvate	<a href="http://www.med.osaka-u.ac.jp/pub/ped/www/study/st09_brain_3_kenkyu_02.html">http://www.med.osaka-u.ac.jp/pub/ped/www/study/st09_brain_3_kenkyu_02.html</a>	Mitochondrial Disorder	Clinical study	Osaka University
L-arginine (JMA-IIA00023)	<a href="https://dbcentre3.jmacct.med.or.jp/jmactr/default.aspx?JMACCTID=JMA-IIA00023">https://dbcentre3.jmacct.med.or.jp/jmactr/default.aspx?JMACCTID=JMA-IIA00023</a>	MELAS	Investigator-initiated clinical trial Phase 3 (Application preparation)	Kurume University School of Medicine
L-arginine (JMA-IIA00025)	<a href="https://dbcentre3.jmacct.med.or.jp/jmactr/default.aspx?JMACCTID=JMA-IIA00025">https://dbcentre3.jmacct.med.or.jp/jmactr/default.aspx?JMACCTID=JMA-IIA00025</a>	MELAS	Investigator-initiated clinical trial Phase 3 (Application preparation)	Kurume University School of Medicine
SPP-004 (5-ALA/Fe) (JMA-IIA00214)	<a href="https://dbcentre3.jmacct.med.or.jp/JMACTR/App/JMACTRE02_04/JMACTRE02_04.aspx?kbn=3&amp;segno=51">https://dbcentre3.jmacct.med.or.jp/JMACTR/App/JMACTRE02_04/JMACTRE02_04.aspx?kbn=3&amp;segno=51</a>	Mitochondrial Disease, mainly to cranial nerve symptoms	Investigator-initiated clinical trial Phase 2	Saitama Medical University Hospital
Idebenone (UMIN000017939)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000020747">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000020747</a>	LHON	Clinical study Phase 1/2	Hyogo College of Medicine
Skin electrical stimulation (UMIN000031057)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000035434">https://upload.umin.ac.jp/cgi-bin/ctr_e/ctr_view.cgi?recptno=R000035434</a>	LHON	Clinical study Phase 1	Kobe University

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# Clinical research, clinical study, and clinical trial around the world

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Compound		Type of disease	Status	Sponsor	Country
Vatiquinone (EPI-743) (NCT02352896)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02352896">https://www.clinicaltrials.gov/ct2/show/NCT02352896</a>	Leigh Syndrome	Clinical trial (DB) Phase 2 (Completed)	Edison Pharmaceuticals Inc	USA
Vatiquinone (EPI-743) (NCT02104336)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02104336">https://www.clinicaltrials.gov/ct2/show/NCT02104336</a>	Pearson Syndrome	Clinical trial Phase 2 (Terminated)	Edison Pharmaceuticals Inc	USA
Vatiquinone (EPI-743) (NCT01642056)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT01642056">https://www.clinicaltrials.gov/ct2/show/NCT01642056</a>	Mitochondrial Disorders, e	Clinical study Phase 2	National Human Genome Research Institute (NHGRI)	USA
Omaveloxolone (RTA408) (NCT02255422)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02255422">https://www.clinicaltrials.gov/ct2/show/NCT02255422</a>	Mitochondrial Myopathy	Clinical trial (DB) Phase 2 (Completed)	Reata Pharmaceuticals Inc	USA Denmark
RP103 (NCT02473445)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02473445">https://www.clinicaltrials.gov/ct2/show/NCT02473445</a>	Mitochondrial Disease	Clinical trial Phase 2 (Terminated)	Horizon Pharma plc (Raptor Pharmaceuticals Inc)	USA
Elamipretide (MTP-131) (NCT03323749)	<a href="https://clinicaltrials.gov/ct2/show/NCT03323749">https://clinicaltrials.gov/ct2/show/NCT03323749</a>	Mitochondrial Myopathy	Clinical trial (DB) Phase 3	Stealth BioTherapeutics Inc	USA
Elamipretide (MTP-131) (NCT02693119)	<a href="https://clinicaltrials.gov/ct2/show/NCT02693119">https://clinicaltrials.gov/ct2/show/NCT02693119</a>	LHON	Clinical trial (DB) Phase 2	Stealth BioTherapeutics Inc	USA
KH176 (NCT02909400)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02909400">https://www.clinicaltrials.gov/ct2/show/NCT02909400</a>	MELAS, MIDD ,etc	Clinical trial (DB) Phase 2 (Completed)	Khondrion BV	Netherlands
GS010 (rAAV2/2-ND4) (NCT02064569) (NCT02652767) (NCT02652780) (NCT03293524) (NCT03406104)	<a href="https://clinicaltrials.gov/ct2/results?term=GS010&amp;Search=Search">https://clinicaltrials.gov/ct2/results?term=GS010&amp;Search=Search</a>	LHON	Clinical trial (DB) Phase 1/2/3	GenSight Biologics	France USA Germany Italy UK
Bezafibrate (NCT02398201)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02398201">https://www.clinicaltrials.gov/ct2/show/NCT02398201</a>	Mitochondrial Myopathy	Clinical study Phase 2 (Completed)	Newcastle-upon-Tyne Hospitals NHS Trust	UK
scAAV2-P1ND4v2 (NCT02161380)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02161380">https://www.clinicaltrials.gov/ct2/show/NCT02161380</a>	LHON	Clinical study Phase 1	University of Miami	USA
rAAV2-ND4 (NCT03153293)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03153293">https://www.clinicaltrials.gov/ct2/show/NCT03153293</a>	LHON	Clinical study Phase 2/3	Huazhong University of Science and Technology	China
Metformin (NCT02500628)	<a href="https://clinicaltrials.gov/ct2/show/NCT02500628">https://clinicaltrials.gov/ct2/show/NCT02500628</a>	Mitochondrial Disease (He	Clinical study Phase 2 (Completed)	Woodinville Psychiatric Associates	USA
L-arginine, Citrulline (NCT02809170)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02809170">https://www.clinicaltrials.gov/ct2/show/NCT02809170</a>	Mitochondrial Disease	Clinical study	Tawam Hospital	UAE
Dapagliflozin (NCT01439854)	<a href="https://clinicaltrials.gov/ct2/show/NCT01439854">https://clinicaltrials.gov/ct2/show/NCT01439854</a>	Multiple Mitochondrial Dysfunctions Syndrome (Carbohydrate Metabolism)	Clinical study (DB)	The University of Texas	USA

Triheptanoin (NCT01379625)	<a href="https://clinicaltrials.gov/ct2/show/NCT01379625">https://clinicaltrials.gov/ct2/show/NCT01379625</a>	Very Long-Chain Acyl-CoA Dehydrogenase (VLCAD) Deficiency, Carnitine Palmitoyltransferase 2 (CPT2) Deficiency, Mitochondrial Trifunctional Protein (TFP) Deficiency, Long-Chain 3 HydroxyacylCoA Dehydrogenase (LCHAD) Deficiency	Clinical study (DB) Phase 2 (Completed)	Oregon Health and Science University	USA
Raxone (Idebenone) (NCT02774005)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02774005">https://www.clinicaltrials.gov/ct2/show/NCT02774005</a>	LHON	Clinical trial Phase 4	Santhera Pharmaceuticals	USA EU
Idebenone (NCT00887562)	<a href="https://clinicaltrials.gov/ct2/show/NCT00887562">https://clinicaltrials.gov/ct2/show/NCT00887562</a>	MELAS	Clinical study (DB) Phase 2 (Completed)	Columbia University	USA
KL1333 (NCT03056209)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03056209">https://www.clinicaltrials.gov/ct2/show/NCT03056209</a>	MELAS Mitochondrial Respiratory Chain	Clinical trial Phase 1 (Completed)	Yungjin Pharm	Korea
Coenzyme Q10 (NCT00432744)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT00432744">https://www.clinicaltrials.gov/ct2/show/NCT00432744</a>	Mitochondrial Disease	Clinical study (DB) Phase 3 (Completed)	University of Florida	USA Canada
Nicotinamide Riboside (NCT03432871)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03432871">https://www.clinicaltrials.gov/ct2/show/NCT03432871</a>	Mitochondrial Disease, etc	Clinical study	Cambridge University	UK
BMSC (NCT03011541)	<a href="https://clinicaltrials.gov/ct2/show/NCT03011541">https://clinicaltrials.gov/ct2/show/NCT03011541</a>	LHON, etc	Clinical trial Phase 2	MD Stem Cells	USA UAE
Allogeneic HSC (NCT02427178)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02427178">https://www.clinicaltrials.gov/ct2/show/NCT02427178</a>	MNGIE	Clinical study Phase 1	Columbia University	USA
CD34+ Cells (NCT03384420)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03384420">https://www.clinicaltrials.gov/ct2/show/NCT03384420</a>	Non-inherited Mitochondrial Disease, Pearson Syndrome	Clinical study Phase 1	Sheba Medical Center	Israel
Icatibant (HOE-140) (NCT03177798)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03177798">https://www.clinicaltrials.gov/ct2/show/NCT03177798</a>	Hemodialysis-Induced Symptom Mitochondrial Diseases	Clinical study (DB) Phase 2	Vanderbilt University	USA

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