

## Approved drugs for Mitochondrial disease

January, 2019

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

January, 2019

Compound		Type of disease	Status	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=R">https://upload.umin.ac.jp/cgi-bin/ctr/ctr.cgi?function=history&amp;action=list&amp;type=summary&amp;recptno=R</a>	MELAS	Clinical study Phase 1	Recruiting	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	Active, not recruiting	Dainippon Sumitomo Pharma Co., Ltd
Taurine (UMIN000029376)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	MELAS	Clinical study Phase 3	Enrolling by invitation	Kawasaki Medical School
Taurine (UMIN000009358)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	MERRF	Clinical study Phase 2	Completed	Kawasaki Medical School
Bezafibrate (UMIN000017984)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	Mitochondrial Fatty Acid Beta-Oxidation Disorder	Clinical study Phase 2/3	Recruiting	Shimane University Hospital
Sodium pyruvate (UMIN000025880)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	Leigh Syndrome	Clinical study Phase 3	Not yet recruiting	Kurume University School of Medicine
Sodium pyruvate (UMIN000025874)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	MELAS/MELA	Clinical study Phase 3	Active, not recruiting	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	Recruiting	Osaka University
L-arginine (JMA-IAA00023)	<a href="https://dbcentre3.jmacct.med.or.jp/jmacctr/default.aspx?JMACCTID=JMA-IAA00023">https://dbcentre3.jmacct.med.or.jp/jmacctr/default.aspx?JMACCTID=JMA-IAA00023</a>	MELAS	Investigator-initiated clinical trial Phase 3	Completed	Kurume University School of Medicine
L-arginine (JMA-IAA00025)	<a href="https://dbcentre3.jmacct.med.or.jp/jmacctr/default.aspx?JMACCTID=JMA-IAA00025">https://dbcentre3.jmacct.med.or.jp/jmacctr/default.aspx?JMACCTID=JMA-IAA00025</a>	MELAS	Investigator-initiated clinical trial Phase 3	Completed	
SPP-004 (5-ALA/Fe) (JMA-IAA00358)	<a href="https://dbcentre3.jmacct.med.or.jp/JMACTR/App/JMACTRE02_04/JMACTRE02_04.aspx?kbn=3&amp;seqno=80">https://dbcentre3.jmacct.med.or.jp/JMACTR/App/JMACTRE02_04/JMACTRE02_04.aspx?kbn=3&amp;seqno=80</a>	Mitochondrial Disease, mainly to cranial nerve symptoms	Investigator-initiated clinical trial (DB) Phase 3	Recruiting	Saitama Medical University Hospital
Idebenone (UMIN000017939)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	LHON	Clinical study Phase 1/2	Active, not recruiting	Hyogo College of Medicine
Skin electrical stimulation (UMIN000031057)	<a href="https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00">https://upload.umin.ac.jp/cgi-bin/ctr/e/ctr_view.cgi?recptno=R00</a>	LHON	Clinical study Phase 1	Recruiting	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	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	Active, not recruiting	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) (NCT01370447)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT01370447">https://www.clinicaltrials.gov/ct2/show/NCT01370447</a>	Mitochondrial Disease	Clinical trial Phase 2	Active, not recruiting	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,	Clinical study (DB) Phase 2	Active, not recruiting	National Human Genome Research Institute (NHGR)	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 Inc (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	Recruiting	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	Active, not recruiting	Stealth BioTherapeutics Inc	USA
Elamipretide (MTP-131) (NCT02805790)	<a href="https://clinicaltrials.gov/ct2/show/NCT02805790">https://clinicaltrials.gov/ct2/show/NCT02805790</a>	Mitochondrial Disease	Clinical trial (DB) Phase 2	Completed	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
Nab-rapamycin(ABI-009) (NCT03747328)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03747328">https://www.clinicaltrials.gov/ct2/show/NCT03747328</a>	Leigh Syndrome	Clinical trial Phase 2	Not yet recruiting	Aadi, LLC	USA
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	Recruiting (NCT03293524) (NCT03406104)	GenSight Biologics	France USA Germany Italy UK
GS010 (rAAV2/2-ND4) (NCT03672968)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03672968">https://www.clinicaltrials.gov/ct2/show/NCT03672968</a>	LHON	Expanded Access (Compassionate use)	Available	GenSight Biologics	USA
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
Bezafibrate (2012-002692-34)	<a href="https://www.clinicaltrialsregister.eu/ctr-search/trial/2012-002692-34">https://www.clinicaltrialsregister.eu/ctr-search/trial/2012-002692-34</a>	Mitochondrial Myopathy	Clinical study Phase 2	Active	Universitätsklinik für Kinder- und Jugendheilkunde	Austria
scAAV2-PIND4v2 (NCT02161380)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02161380">https://www.clinicaltrials.gov/ct2/show/NCT02161380</a>	LHON	Clinical study Phase 1	Recruiting	University of Miami	USA
rAAV2-ND4 (NCT03428178)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03428178">https://www.clinicaltrials.gov/ct2/show/NCT03428178</a>	LHON	Clinical study	Recruiting	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 (H	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	Active, not recruiting	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)	Completed	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
Triheptanoin (NCT01461304)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT01461304">https://www.clinicaltrials.gov/ct2/show/NCT01461304</a>	Very Long-chain acylCoA Dehydrogenase (VLCAD) Deficiency, Carnitine Palmitoyltransferase Deficiencies (CPT1, CPT2), Mitochondrial Trifunctional Protein Deficiency, Long-chain Hydroxyacyl-CoA Dehydrogenase Deficiency, Pyruvate Carboxylase Deficiency Disease, ACYL-CoA DEHYDROGENASE FAMILY, MEMBER 9, DEFICIENCY of Barth Syndrome,	Expanded Access (Compassionate use)	Available	University of Pittsburgh	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	Recruiting	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	Recruiting	Cambridge University	UK
Resveratrol (NCT03728777)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03728777">https://www.clinicaltrials.gov/ct2/show/NCT03728777</a>	Mitochondrial Myopathy, etc	Clinical study (DB)	Recruiting	Rigshospitalet	Denmark
BMSC (NCT03011541)	<a href="https://clinicaltrials.gov/ct2/show/NCT03011541">https://clinicaltrials.gov/ct2/show/NCT03011541</a>	LHON, etc	Clinical trial	Recruiting	MD Stem Cells	USA UAE
Allogenic HSC (NCT02427178)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02427178">https://www.clinicaltrials.gov/ct2/show/NCT02427178</a>	MNGIE	Clinical study Phase 1	Recruiting	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	Not yet recruiting	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	Active, not recruiting	Vanderbilt University	USA
Carbaglu (NCT01599286)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT01599286">https://www.clinicaltrials.gov/ct2/show/NCT01599286</a>	Propionic Acidemia, Carbamoyl-Phosphate Synthase I Deficiency Disease, etc	Clinical study (DB) Phase 2	Recruiting	Children's Research Institute	USA
Dichloroacetate (NCT02616484)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT02616484">https://www.clinicaltrials.gov/ct2/show/NCT02616484</a>	Pyruvate Dehydrogenase Complex Deficiency	Clinical study (DB) Phase 3	Recruiting	University of Florida	USA
Sodium Phenylbutyrate (NCT03734263)	<a href="https://www.clinicaltrials.gov/ct2/show/NCT03734263">https://www.clinicaltrials.gov/ct2/show/NCT03734263</a>	Pyruvate Dehydrogenase Complex Deficiency	Clinical trial Phase 1/2	Recruiting	Fondazione Telethon	Italia