

Assisted delivery of antisense therapeutics in animal models of  
heritable neurodegenerative and neuromuscular disorders: a  
systematic review and meta-analysis

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## File S1. Reference list of the included studies.

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## **PRISMA 2009 Checklist**

**File S2. PRISMA checklist.**



# PRISMA 2009 Checklist

| Section/topic                      | #  | Checklist item  | Reported on page #            |
|------------------------------------|----|---|-------------------------------|
| <b>TITLE</b>                       |    |   |                               |
| Title                              | 1  | Identify the report as a systematic review, meta-analysis, or both.   | 1                             |
| <b>ABSTRACT</b>                    |    |   |                               |
| Structured summary                 | 2  | Provide a structured summary including, as applicable: background; objectives; data sources; study eligibility criteria, participants, and interventions; study appraisal and synthesis methods; results; limitations; conclusions and implications of key findings; systematic review registration number. | 2                             |
| <b>INTRODUCTION</b>                |    |   |                               |
| Rationale                          | 3  | Describe the rationale for the review in the context of what is already known.  | 3-7                           |
| Objectives                         | 4  | Provide an explicit statement of questions being addressed with reference to participants, interventions, comparisons, outcomes, and study design (PICOS).  | 7                             |
| <b>METHODS</b>                     |    |   |                               |
| Protocol and registration          | 5  | Indicate if a review protocol exists, if and where it can be accessed (e.g., Web address), and, if available, provide registration information including registration number.   | 24                            |
| Eligibility criteria               | 6  | Specify study characteristics (e.g., PICOS, length of follow-up) and report characteristics (e.g., years considered, language, publication status) used as criteria for eligibility, giving rationale.  | 24                            |
| Information sources                | 7  | Describe all information sources (e.g., databases with dates of coverage, contact with study authors to identify additional studies) in the search and date last searched.  | 24                            |
| Search                             | 8  | Present full electronic search strategy for at least one database, including any limits used, such that it could be repeated.   | Supplementary File S3         |
| Study selection                    | 9  | State the process for selecting studies (i.e., screening, eligibility, included in systematic review, and, if applicable, included in the meta-analysis).   | 24-25                         |
| Data collection process            | 10 | Describe method of data extraction from reports (e.g., piloted forms, independently, in duplicate) and any processes for obtaining and confirming data from investigators.  | 25                            |
| Data items                         | 11 | List and define all variables for which data were sought (e.g., PICOS, funding sources) and any assumptions and simplifications made.   | 25-27; Supplementary Table S1 |
| Risk of bias in individual studies | 12 | Describe methods used for assessing risk of bias of individual studies (including specification of whether this was done at the study or outcome level), and how this information is to be used in any data synthesis.  | 25; Supplementary Table S2    |
| Summary measures                   | 13 | State the principal summary measures (e.g., risk ratio, difference in means).   | 26-27                         |
| Synthesis of results               | 14 | Describe the methods of handling data and combining results of studies, if done, including measures of consistency (e.g., $I^2$ ) for each meta-analysis.   | 27                            |



# PRISMA 2009 Checklist

| Section/topic                 | #  | Checklist item   | Reported on page #           |
|-------------------------------|----|--|------------------------------|
| Risk of bias across studies   | 15 | Specify any assessment of risk of bias that may affect the cumulative evidence (e.g., publication bias, selective reporting within studies).   | 25                           |
| Additional analyses           | 16 | Describe methods of additional analyses (e.g., sensitivity or subgroup analyses, meta-regression), if done, indicating which were pre-specified.   | -                            |
| <b>RESULTS</b>                |    |  |                              |
| Study selection               | 17 | Give numbers of studies screened, assessed for eligibility, and included in the review, with reasons for exclusions at each stage, ideally with a flow diagram.  | 8; Fig 1                     |
| Study characteristics         | 18 | For each study, present characteristics for which data were extracted (e.g., study size, PICOS, follow-up period) and provide the citations.   | 8-14; Supplementary Table S1 |
| Risk of bias within studies   | 19 | Present data on risk of bias of each study and, if available, any outcome level assessment (see item 12).  | 15; Supplementary Table S2   |
| Results of individual studies | 20 | For all outcomes considered (benefits or harms), present, for each study: (a) simple summary data for each intervention group (b) effect estimates and confidence intervals, ideally with a forest plot. | 15-16; Fig 6                 |
| Synthesis of results          | 21 | Present results of each meta-analysis done, including confidence intervals and measures of consistency.  | 15-16                        |
| Risk of bias across studies   | 22 | Present results of any assessment of risk of bias across studies (see Item 15).  | 15                           |
| Additional analysis           | 23 | Give results of additional analyses, if done (e.g., sensitivity or subgroup analyses, meta-regression [see Item 16]).  | -                            |
| <b>DISCUSSION</b>             |    |  |                              |
| Summary of evidence           | 24 | Summarize the main findings including the strength of evidence for each main outcome; consider their relevance to key groups (e.g., healthcare providers, users, and policy makers).                     | 17-22                        |
| Limitations                   | 25 | Discuss limitations at study and outcome level (e.g., risk of bias), and at review-level (e.g., incomplete retrieval of identified research, reporting bias).  | 17-22                        |
| Conclusions                   | 26 | Provide a general interpretation of the results in the context of other evidence, and implications for future research.  | 23                           |
| <b>FUNDING</b>                |    |  |                              |
| Funding                       | 27 | Describe sources of funding for the systematic review and other support (e.g., supply of data); role of funders for the systematic review.   | 36                           |

From: Moher D, Liberati A, Tetzlaff J, Altman DG, The PRISMA Group (2009). Preferred Reporting Items for Systematic Reviews and Meta-Analyses: The PRISMA Statement. PLoS Med 6(7): e1000097. doi:10.1371/journal.pmed1000097

For more information, visit: [www.prisma-statement.org](http://www.prisma-statement.org).

# File S3. Full search strategies for Embase, Pubmed and Web of Science

## 1. Embase

### Search component #1: Intervention

((cell penetrating peptide/ OR exp drug carrier/ OR liposomal delivery/ OR exp nanoparticle/ OR nanopharmaceutics/) OR (AAV OR Adeno-associated OR Cationic lipid OR Cationic lipids OR Cell-penetrating peptide OR Cell-penetrating peptides OR Chitosan OR Conjugate OR Conjugated OR Conjugates OR Conjugation OR Copolymer OR Co-polymer OR Co-polymers OR Copolymers OR CPP OR CPPs OR Dendrimer OR Dendrimers OR Dendriplex OR Dendriplexes OR Excipient OR Excipients OR Formulation OR Formulations OR Lipid OR Lipids OR Lipofectamine OR Lipofection OR Lipoplex OR Lipoplexes OR liposome OR liposomes OR Nanoballs OR Nanocapsule OR Nanocapsules OR Nanocarrier OR Nanocarriers OR Nanocomposite OR Nanocomposites OR Nanoconjugate OR Nanoconjugates OR Nanocrystal OR Nanocrystals OR nanoparticle OR nanoparticles OR Nanoparticulate OR Nanorod OR Nanorods OR Nanotube OR Nanotubes OR Nano-ball OR Nano-balls OR Nano-capsule OR Nano-capsules OR Nano-carrier OR Nano-carriers OR Nano-composite OR Nano-composites OR Nano-conjugate OR Nano-conjugates OR Nano-crystal OR Nano-crystals OR Nanoparticle OR Nano-particles OR Nano-particulate OR Nano-rod OR Nano-rods OR Nano-tube OR Nanotubes OR Oligoplex OR Oligoplexes OR PEI OR peptide OR peptide-linked OR peptides OR PLGA OR Polyelectrolyte OR Polyelectrolytes OR Polyethylenimine OR polylactic-co-glycolic acid OR Polymer OR Polymers OR Polymersomes OR Polyplex OR Polyplexes OR Protamine OR Silica OR Thiomers OR Thiomers OR Transfection OR Vector OR Vectorized OR Vectors OR Viral OR Virosome OR Virosomes OR Virus OR Viruses OR Virus-like particle OR Virus-like particles OR VLP OR VLPs).ti,ab,kw.) AND ((exp antisense oligonucleotide/ OR locked nucleic acid/ OR morpholino oligonucleotide/ OR oligonucleotide phosphorothioate/ OR peptide nucleic acid/) OR (antisense OR AON OR AONs OR ASO OR ASOs OR LNA OR LNAs OR Locked nucleic acid OR Locked nucleic acids OR MORF OR MORFs OR Morpholino OR Morpholinos OR Peptide nucleic acid OR Peptide nucleic acids OR Phosphorothioate OR Phosphorothioates OR PMO OR PMOs OR PNA OR PNAs OR SCO OR SCOs OR splice-correcting oligonucleotide OR splice-correcting oligonucleotides OR splice-switching oligonucleotide OR splice-switching oligonucleotides OR SSO OR SSOs).ti,ab,kw.)

### Search component #2: Disease

(adrenoleukodystrophy/ OR Alexander disease/ OR Alstrom syndrome/ OR Alzheimer Disease/ OR amyloid neuropathy/ OR amyotrophic lateral sclerosis/ OR Ataxia Telangiectasia/ OR becker muscular dystrophy/ OR bulbar paralysis/ OR Canavan disease/ OR central core disease/ OR cerebellum dyssynergia/ OR chronic progressive external ophthalmoplegia/ OR Cockayne syndrome/ OR congenital analgesia/ OR diffuse Lewy body disease/ OR distal myopathy/ OR dysautonomia/ OR fatal familial insomnia/ OR Friedreich ataxia/ OR frontotemporal dementia/ OR Gerstmann Straussler Scheinker syndrome/ OR giant axonal neuropathy/ OR Gilles de la Tourette syndrome/ OR globoid cell leukodystrophy/ OR glycogen storage disease type 7/ OR hereditary motor sensory neuropathy/ OR hereditary optic atrophy/ OR Huntington chorea/ OR hypokalemic periodic paralysis/ OR inclusion body myositis/ OR Kearns Sayre syndrome/ OR Kennedy disease/ OR Luft disease/ OR Machado Joseph disease/ OR MELAS syndrome/ OR MERRF syndrome/ OR metachromatic leukodystrophy/ OR mitochondrial encephalomyopathy/ OR mitochondrial myopathy/ OR Motor Neuron Disease/ OR muscular dystrophy/ OR Myasthenia Gravis/ OR myoclonus epilepsy/ OR myokymia/ OR Myositis/ OR myotonic dystrophy/ OR nemaline myopathy/ OR neural ceroid lipofuscinosis/ OR neurodegeneration with brain

iron accumulation/ OR neurofibromatosis/ OR olivopontocerebellar atrophy/ OR ossifying myositis/ OR Parkinson Disease/ OR Pelizaeus Merzbacher disease/ OR periodic paralysis/ OR peripheral neuropathy/ OR Pick presenile dementia/ OR polyneuropathy/ OR progressive nonfluent aphasia/ OR Refsum disease/ OR sarcoglycanopathy/ OR Shy Drager syndrome/ OR spinal muscular atrophy/ OR spinocerebellar degeneration/ OR striatonigral degeneration/ OR Tangier disease/ OR tauopathy/ OR TDP 43 proteinopathy/ OR Thomsen disease/ OR torsion dystonia/ OR tuberous sclerosis/ OR Wilson disease/ OR Wolfram syndrome/ OR congenital myasthenic syndrome/) OR (((autosomal OR congenital OR dominant OR familial OR genealogical OR genetic OR hereditary OR heritable OR inborn OR inheritable OR inheritance OR inherited OR recessive OR single-gene OR X-linked).ti,ab,kw.) AND (((degenerative AND nervous system) OR (degenerative AND neurologic) OR (degenerative AND neurological) OR (degenerative AND spinal cord) OR fasciculation OR neurodegenerative OR neuromuscular).ti,ab,kw.)) OR ("Adenoma Sebaceum" OR "adreno leucodystrophy" OR "adreno leukodystrophy" OR "adrenoleucodystrophy" OR "Adrenoleukodystrophy" OR "adrenoleukomyeloneuropathy" OR "adrenomyeloneuropathy" OR "Alexander Disease" OR "alexander syndrome" OR "Alexanders Disease" OR "Alexander's Disease" OR "Alstrom Hallgren Syndrome" OR "Alstrom Syndrome" OR "Alstrom-Hallgren Syndrome" OR "Alstroms Syndrome" OR "Alstrom's Syndrome" OR "Alzheimer's" OR "Alzheimer" OR "Alzheimers" OR "amaurotic familial idiocy" OR "amaurotic idiocy" OR "Aminoacylase 2 Deficiency" OR "amyloid neuropathies" OR "Amyloid Neuropathy" OR "Amyloid Polyneuropathies" OR "amyloid polyneuropathy" OR "amyloidotic neuropathy" OR "amyloidotic polyneuropathy" OR "Amyotrophic Lateral Sclerosis" OR "analgesia congenita" OR "Analphalipoproteinaemia" OR "Analphalipoproteinemia" OR "Anterior Horn Cell Disease" OR "ARSA Deficiency" OR "Arylsulfatase A Deficiencies" OR "Arylsulfatase A Deficiency" OR "ASP Deficiency" OR "ASPA Deficiency" OR "Aspartoacylase Deficiency" OR "Ataxia Telangiectasia Syndrome" OR "Ataxia Telangiectasia" OR "ataxia telangiectatica" OR "ataxia teleangiectasia" OR "Autosomal Dominant Cerebellar Ataxia, Type II" OR "Autosomal Dominant Optic Atrophy" OR "Autosomal Dominant Spastic Paraplegia" OR "Autosomal Dominant Striatonigral Degeneration" OR "Autosomal Recessive Spastic Paraplegia" OR "Azorean Disease" OR "balo disease" OR "Baltic Myoclonus" OR "bar syndrome" OR "Batten" OR "Batten-Turner" OR "Becker Disease" OR "Becker Muscular Dystrophy" OR "Becker's Muscular Dystrophy" OR "beta-Galactosidase Deficiencies" OR "beta-Galactosidase Deficiency" OR "Bilateral Acoustic Neurinoma" OR "Bilateral Acoustic Neurinomas" OR "Bilateral Acoustic Neuroma" OR "Bilateral Acoustic Neuromas" OR "Bilateral Acoustic Schwannoma" OR "Bilateral Acoustic Schwannomas" OR "bourneville disease" OR "Bourneville Phakomatosis" OR "bourneville pringle syndrome" OR "Bourneville Pringle's Disease" OR "bourneville syndrome" OR "bourneville tuberous sclerosis" OR "Bourneville-Pringle Disease" OR "Bourneville-Pringles Disease" OR "Bourneville-Pringle's Disease" OR "Bourneville's Disease" OR "Bourneville's Syndrome" OR "brain sclerosis" OR "bulbar and spinal muscular atrophy" OR "bulbar dysfunction" OR "bulbar muscle weakness" OR "Bulbar palsies" OR "Bulbar Palsy" OR "bulbar paralysis" OR "bulbar sign" OR "bulbar symptom" OR "bulbar syndrome" OR "bulbar weakness" OR "Bulbospinal atrophy" OR "Bulbo-Spinal Atrophy" OR "bulbospinal muscular atrophy" OR "bulbo-spinal muscular atrophy" OR "Bulbospinal Neuronopathy" OR "Canavan Disease" OR "canavan syndrome" OR "Canavan's disease" OR "Canavan-van Bogaert-Bertrand Disease" OR "Central core disease" OR "Central Core Diseases" OR "Central Core Myopathies" OR "Central Core Myopathy" OR "Cerebellar Degeneration with Slow Eye Movements" OR "cerebellar dyssynergia" OR "Cerebral Scleroses" OR "cerebral sclerosis" OR "Cerebroside Sulphatase Deficiency" OR "Ceroid Storage Disease" OR "Ceroid Storage Diseases" OR "Channelopathy-Associated Insensitivity To Pain" OR "Charcot Disease" OR "charcot marie disease" OR "Charcot Marie Tooth" OR "Childhood Torsion Disease" OR "Cholesterol Thesaurismosis" OR "chorea major" OR "chronic progressive chorea" OR "Chronic Progressive External Ophthalmoplegia" OR "Chronic Progressive Hereditary Chorea" OR "Circumscribed Lobar Atrophy of the Brain" OR "Cockayne Syndrome" OR "Cockayne" OR "Cockayne's" OR "concentric sclerosis" OR "congenital anaesthesia" OR "congenital analgesia" OR "congenital anesthesia" OR "congenital atonic sclerotic muscular dystrophy" OR "congenital generalized analgesia" OR "congenital indifference to pain" OR "congenital insensitivity to pain" OR "Congenital Myopathy" OR "Congenital Myotonia" OR "congenital pain indifference" OR "Congenital Pain Insensitivity" OR

"congenital paramyotonia" OR "Continuous Muscle Activity Syndrome" OR "Copper Storage Disease" OR "CPEO with Ragged Red Fibers" OR "cranial nerve paralysis" OR "DDPAC" OR "degeneratio hepato lenticularis" OR "dejerine sottas" OR "Dejerine-Thomas" OR "dementia with Lewy bodies" OR "dementia with Lewy body" OR "Dementia with Lobar Atrophy and Neuronal Cytoplasmic Inclusions" OR "DIDMOAD" OR "Dilated Cardiomyopathy" OR "Disinhibition-Dementia-Parkinsonism-Amyotrophy" OR "Distal 1 Myopathy" OR "Distal Muscular Dystrophies" OR "Distal Muscular Dystrophy" OR "Distal Myopathies" OR "Distal Myopathy" OR "Dominant Hereditary Sensory Neuropathy" OR "Dominant Optic Atrophies" OR "dominant optic atrophy" OR "Duchenne" OR "dysantonomia" OR "Dysmyelinogenic Leukodystrophy" OR "Dyssynergia Cerebellaris " OR "Dystonia Musculorum Deformans" OR "dystrophia myotonica" OR "dystrophic myotonia" OR "Emery-Dreifuss" OR "encephalitis periaxialis" OR "epiloia" OR "Eulenburg" OR "Faciocapulohumeral" OR "Facio-Scapulo-Humeral" OR "Faciocapuloperoneal" OR "familial autonomic dysfunction" OR "Familial dysautonomia" OR "familial dysautonomy" OR "Familial Fatal Insomnia" OR "Familial Periodic Paralysis" OR "Familial Periodic Paralysis" OR "Familial Polyneuropathies" OR "Familial Polyneuropathy" OR "familial spastic paralysis" OR "familial spastic paraparesis" OR "familial spastic paraplegia" OR "Fatal Familial Insomnia" OR "Fazio-Londe Disease" OR "Fazio-Londe Syndrome" OR "Fibrinoid Degeneration of Astrocytes" OR "Fibrodysplasia Ossificans" OR "Friedreich Ataxia" OR "Friedreich" OR "Friedreich's" OR "Friedreichs" OR "frontal dementia" OR "frontal lobe dementia" OR "Frontotemporal Dementia" OR "frontotemporal dementias" OR "Frontotemporal Lobar Degeneration" OR "Frontotemporal Lobe Dementia" OR "FTD" OR "FTDP" OR "FTLD" OR "Fukuhara disease" OR "Galactocerebrosidase Deficiency" OR "Galactosylceramidase Deficiency" OR "galactosylceramide lipidosis" OR "GALC Deficiency" OR "Gehrig Disease" OR "Gehrigs Disease" OR "Gehrig's Disease" OR "Generalized Myotonia" OR "Generalized Myotonias" OR "Gerstmann-Straussler" OR "Gerstmann-Straussler-Scheinker Disease" OR "Giant Axonal Neuropathy" OR "globoid cell dystrophy" OR "globoid cell leukodystrophy" OR "Globoid Cell Leukodystrophy" OR "Globoid Leukodystrophies" OR "Globoid Leukodystrophy" OR "Glycogen Storage Disease Type VII" OR "Glycogen Storage Disease VII" OR "Graefe's Disease" OR "Greenfield Disease" OR "Greenfield's Disease" OR "GSD VII" OR "GSS" OR "Guam Disease" OR "Hallervorden Spatz" OR "Hallervorden-Spatz" OR "Hauptmann-Thannhauser" OR "HDDD1" OR "HDDD2" OR "hemimegalencephaly" OR "hepatocerebral degeneration" OR "Hepatocerebral Degenerations" OR "Hepatolenticular Degeneration" OR "hepatolenticular syndrome" OR "Hereditary and Autonomic Neuropathy" OR "Hereditary Areflexic Dystasia" OR "hereditary atactic polyneuritis" OR "hereditary chorea" OR "Hereditary Dysphasic Disinhibition Dementia" OR "hereditary motor and sensory neuropathies" OR "Hereditary Motor and Sensory Neuropathy " OR "Hereditary Motor and Sensory Neuropathy 5" OR "Hereditary Motor and Sensory Neuropathy Type IV" OR "Hereditary Motor And Sensory Neuropathy V" OR "Hereditary Motor Neuronopathies" OR "Hereditary Motor Neuronopathy" OR "hereditary motor sensory neuropathy type IV" OR "hereditary motor sensory neuropathy" OR "hereditary myotonia" OR "Hereditary Optic Atrophies" OR "Hereditary Optic Atrophy" OR "hereditary optic nerve atrophy" OR "Hereditary Optic Neuroretinopathy" OR "hereditary sensorimotor neuropathy" OR "Hereditary Sensory and Autonomic Neuropathy" OR "hereditary sensory and motor neuropathy" OR "Hereditary Sensory Neuropathy" OR "hereditary spastic paralysis" OR "hereditary spastic paraparesis" OR "Hereditary Spastic Paraplegia" OR "Hereditary Spastic Paraplegias" OR "hereditary spinal ataxia" OR "Hereditary Spinal Sclerosis" OR "Hereditary Type I Motor and Sensory Neuropathy" OR "heredopathia atactica polyneuritiformis" OR "High Density Lipoprotein Deficiency" OR "HMSN IV" OR "HMSN Type V" OR "HMSN V" OR "HMSN" OR "HOKPP" OR "HSAN III" OR "HSAN Type III" OR "HSAN3" OR "HSN-III" OR "Huntington disease" OR "Huntington" OR "Huntington's" OR "Hyperkalemic Periodic Paralysis" OR "hypokalaemic periodic paralysis" OR "Hypokalemic Periodic Paralysis" OR "HYPOKPP" OR "hypomyelinating Leukodystrophy" OR "HYPOPP" OR "Inclusion Body Myopathy" OR "Inclusion Body Myositides" OR "Inclusion Body Myositis" OR "Infantile Muscular Atrophies" OR "Infantile Muscular Atrophy" OR "Inherited Polyneuropathies" OR "Inherited Polyneuropathy" OR "Inherited Spongiform Encephalopathy" OR "Insomnia Familial Fatal" OR "Isaac Syndrome" OR "Isaacs Mertens Syndrome" OR "Isaacs Syndrome" OR "Isaacs' Syndrome" OR "Isaacs-Mertens Syndrome" OR "Jansky-Bielschowsky" OR "Joseph Azorean Disease" OR "joseph disease" OR "juvenile amaurotic family idiocy" OR "juvenile amaurotic idiocy" OR "Juvenile Cerebroretinal

Degeneration" OR "juvenile chorea" OR "Juvenile Muscular Atrophies" OR "Juvenile Muscular Atrophy" OR "Juvenile Optic Atrophy" OR "Juvenile-Onset Neuroaxonal Dystrophy" OR "Kearn Syndrome" OR "Kearne-Sayre" OR "Kearns syndrome" OR "Kearns' Syndrome" OR "Kearn-Sayre" OR "Kearns-Sayer" OR "Kearns-Sayre Syndrome" OR "Kearns-Sayre" OR "Kennedy Disease" OR "Kennedy Syndrome" OR "Kennedys Disease" OR "Kennedy's Disease" OR "Kennedy's syndrome" OR "Kjer Optic Atrophy" OR "Kjers Optic Atrophy" OR "Kjer's Optic Atrophy" OR "Kjer-Type Optic Atrophy" OR "Krabbe" OR "Krabbes" OR "Krabbe's" OR "Kuf Disease" OR "Kufs Disease" OR "Kufs Disease" OR "Kugelberg-Welander" OR "Lafora Disease" OR "Lafora" OR "Landouzy-Dejerine" OR "Lateral Scleroses" OR "Lateral Sclerosis" OR "Leber Disease" OR "Leber Hereditary Optic Atrophy" OR "Leber Hereditary optic atrophy" OR "Leber Hereditary optic neuropathy" OR "Leber optic atrophy" OR "Leber Optic Neuropathy" OR "Lebers Disease" OR "Leber's Disease" OR "Leber's Diseases" OR "Leber's Hereditary Optic Atrophy" OR "Leber's Hereditary Optic Neuropathy" OR "Leber's Optic Atrophy" OR "Lebers Optic Neuropathy" OR "Leber's Optic Neuropathy" OR "Leukodystrophy with Rosenthal Fibers" OR "Lewy body dementia" OR "Lewy body dementias" OR "Lewy Body Disease" OR "Lewy body diseases" OR "Limb-Girdle" OR "Lipofuscin Storage Disease" OR "Lobar Atrophy Of Brain" OR "Lobar Atrophy of the Brain" OR "Luft Disease" OR "Lufts Disease" OR "Luft's Disease" OR "lundborg syndrome" OR "machado disease" OR "Machado-Joseph Disease" OR "Machado-Joseph" OR "macrencephaly" OR "Megaconial Myopathies" OR "Megaconial Myopathy" OR "megalencephalic leukodystrophy" OR "megalencephaly" OR "melanodermic leukodystrophy" OR "MELAS Syndrome" OR "MELAS" OR "Menzel Type OPCA" OR "MERRF Syndrome" OR "MERRF" OR "metachromatic leucodystrophy" OR "metachromatic leucoencephalopathy" OR "Metachromatic Leukodystrophies" OR "Metachromatic Leukodystrophy" OR "metachromatic leucoencephalopathy" OR "Mitochondrial Encephalomyopathies" OR "Mitochondrial Encephalomyopathy" OR "Mitochondrial Myopathies" OR "Mitochondrial Myopathy" OR "Mitochondrial Ocular Myopathy" OR "Mitochondrial-Encephalopathy-Lactic Acidosis-Stroke" OR "Molluscum Fibrosum" OR "morbus wilson" OR "motoneuron disease" OR "Motor Neuron Disease" OR "Motor Neuron Diseases" OR "motor neurone disease" OR "motor system disease" OR "Motor System Diseases" OR "multiple mucosal neuroma" OR "multiple neurofibroma" OR "Multiple Neurofibromas" OR "multiple neuroma" OR "Multiple System Atrophies" OR "Multiple System Atrophy" OR "Multiple System Tauopathy with Presenile Dementia" OR "Multisystem Atrophies" OR "Multisystem Atrophy" OR "Multisystemic Atrophies" OR "Multisystemic Atrophy" OR "muscle dystrophia" OR "muscle dystrophy" OR "Muscle Phosphofructokinase Deficiency" OR "muscular dystrophia" OR "Muscular Dystrophies" OR "Muscular Dystrophy" OR "Myelopathic Muscular Atrophy" OR "Myoclonic Cerebellar Dyssynergia" OR "myoclonic epilepsies" OR "myoclonic epilepsy" OR "Myoclonus Epilepsies" OR "Myoclonus epilepsy" OR "myodystrophia" OR "Myodystrophies" OR "myodystrophy" OR "Myokymia" OR "Myokymias" OR "myositis calcificans" OR "Myositis Ossificans" OR "myotonia atrophica" OR "Myotonia Congenita" OR "myotonia dystrophica" OR "myotonia hereditaria" OR "Myotonia Levior" OR "myotonic atrophy" OR "myotonic dystrophia" OR "Myotonic Dystrophies" OR "Myotonic Dystrophy" OR "myotonic muscle dystrophy" OR "myotonic muscular dystrophy" OR "NBIA" OR "Nemaline Myopathies" OR "Nemaline Myopathy" OR "Nemaline Rod Disease" OR "neurodegeneration with brain iron accumulation" OR "neurofibromatoses" OR "Neurofibromatosis" OR "neurogenic amyotrophy" OR "neurogenic atrophy" OR "neurogenic muscle atrophy" OR "neurogenic muscular atrophy" OR "neurogenic osteoarthropathy" OR "neurogenic para osteoarthropathy" OR "neurogenic paraosteoarthropathy" OR "Neurogenic Scapuloperoneal Amyotrophy" OR "neuroinomatosis" OR "neuromatosis" OR "neuromyotonia" OR "Neuronal Ceroid-Lipofuscinoses" OR "Neuronal Ceroid-Lipofuscinosis" OR "neuronal ceroidosis" OR "Neuropathic Amyloidosis" OR "Nigrospinothal Degeneration" OR "Non fluent Primary Progressive Aphasia" OR "Non-fluent Primary Progressive Aphasia" OR "Nonfluent progressive aphasia" OR "Non-fluent progressive aphasia" OR "Normokalemic Periodic Paralysis" OR "Ocular Muscular Dystrophy" OR "oculocranosomatic disease" OR "oculocranosomatic syndrome" OR "Oculocranosomatic Syndromes" OR "Oculopharyngeal" OR "olivocerebellar atrophy" OR "Olivopontocerebellar Atrophies" OR "Olivopontocerebellar Atrophy" OR "Olivopontocerebellar" OR "Olivo-Ponto-Cerebellar" OR "OPCA with Retinal Degeneration" OR "Ophthalmoplegia Plus Syndrome" OR "Ophthalmoplegia Plus Syndromes" OR "Optic Atrophy 1" OR "optic atrophy type 1" OR "ossifying fibrodysplasia" OR "Ossifying myositis" OR

"osteoplastic myopathy" OR "Pantothenate Kinase Associated Neurodegeneration" OR "Pantothenate Kinase-Associated Neurodegeneration" OR "paralysis agitans" OR "paramyotonia congenita" OR "Parkinson" OR "Parkinson's" OR "Pelizaeus Merzbacher" OR "Pelizaeus-Merzbacher Disease" OR "periodic hypokalaemic paralysis" OR "periodic hypokalemic paralysis" OR "Periodic Paralysis Hypokalemic" OR "Periodic Paralysis- Hypokalemic" OR "peroneal atrophy" OR "peroneal muscle atrophy" OR "Peroneal Muscular Atrophies" OR "Peroneal Muscular Atrophy with Pyramidal Features" OR "peroneal muscular atrophy" OR "peroneal progressive muscular atrophy" OR "PfkM Deficiency" OR "Phytanic Acid Oxidase Deficiency" OR "phytanic acid storage disease" OR "Pick complex" OR "Pick dementia" OR "Pick Disease" OR "Pick presenile dementia" OR "Pick syndrome" OR "Pick's complex" OR "Picks Disease" OR "Pick's disease" OR "Pigmentary Pallidal Degeneration" OR "PKAN Neuroaxonal Dystrophy" OR "Pleconial Myopathy" OR "PNFA" OR "Polyneuritic Amyloidosis" OR "PPA Syndrome" OR "PPA Syndromes" OR "Presenile Dementia" OR "Primary Adhalinopathies" OR "Primary Adhalinopathy" OR "primary dysautonomia" OR "primary dysautonomias" OR "Primary Parkinsonism" OR "Primary Progressive Nonfluent Aphasia" OR "Primary Senile Degenerative Dementia" OR "pringle bourneville disease" OR "pringle disease" OR "pringle syndrome" OR "Progeria-Like Syndrome" OR "Progeria-Like Syndromes" OR "progressive external ophthalmoplegia" OR "progressive lenticular degeneration" OR "Progressive Muscular Atrophies" OR "Progressive Muscular Atrophy" OR "Progressive nonfluent aphasia" OR "Progressive non-fluent aphasia" OR "PROMM" OR "Proximal Myotonic Myopathies" OR "proximal myotonic myopathy" OR "Pseudohypertrophic Muscular Dystrophy" OR "Pseudomyotonia" OR "Pseudosclerosis" OR "Psychosine Lipidosis" OR "Quantal Squander" OR "Ramsay Hunt " OR "Recklinghausen disease" OR "Recklinghausens Disease" OR "Recklinghausen's Disease" OR "Refsum Disease" OR "Refsum" OR "Refsum's" OR "Refsums" OR "Ricker" OR "Riley-Day Syndrome" OR "Rod Myopathies" OR "Rod Myopathy" OR "Rod-Body Myopathy" OR "Roussy Levy" OR "Santavuori-Haltia" OR "Sarcoglycanopathies" OR "Sarcoglycanopathy" OR "SBMA" OR "SCA1" OR "SCA1s" OR "SCA2" OR "SCA3" OR "SCA4" OR "SCA5" OR "SCA6" OR "SCA7" OR "Scapulo-ilio-peroneal" OR "Scapulooperoneal" OR "Sclerosis Tuberosa" OR "sebaceous adenoma" OR "Semantic Dementia" OR "Semantic Dementias" OR "Senile Dementia" OR "Shy Drager syndrome" OR "Spastic Paraplegia 2" OR "Spastic Paraplegia Type 2" OR "Spielmeyer-Sjogren" OR "Spielmeyer-Vogt" OR "Spinal Amyotrophies" OR "Spinal Amyotrophy" OR "spinal and bulbar muscular atrophy" OR "spinal heredoataxia" OR "spinal muscle atrophy" OR "Spinal Muscular Atrophy" OR "spine muscle atrophy" OR "spinobulbar muscular atrophy" OR "Spinocerebellar Ataxia" OR "Spinocerebellar Ataxias" OR "Spinocerebellar Atrophies" OR "spinocerebellar atrophy" OR "Spinocerebellar Degeneration" OR "spinocerebellar degenerations" OR "Spongiform Leukodystrophy" OR "Spongy Degeneration" OR "Spongy Disease " OR "Steinert" OR "Steinerts" OR "Steinert's" OR "Struempell Lorrain" OR "Struempell" OR "Strumpel" OR "Strumpell Lorrain" OR "Strumpell" OR "Strumpell's" OR "Strumpel's" OR "sulfatide lipidosis" OR "sulfatidosis" OR "sulphatide lipidosis" OR "Syndrome of Continuous Muscle Activity" OR "Tangier Disease" OR "tarui disease" OR "Taruis Disease" OR "Tarui's Disease" OR "TDP 43 pathology" OR "TDP 43 proteinopathies" OR "TDP-43 Proteinopathy" OR "Thomsen Disease" OR "Thomsen myotonia" OR "Thomsens Disease" OR "Thomsen's Disease" OR "Thomsens myotonia" OR "Thomsen's myotonia" OR "Tibial Muscular Dystrophy" OR "Torsion dystonia" OR "Torsion Dystonias" OR "torsion spasm" OR "Tourette Syndrome" OR "Tourette" OR "Tourette's" OR "Tourettes" OR "tuberose sclerosis" OR "Tuberous Sclerosis" OR "Udd Myopathy" OR "Unverricht Disease" OR "Unverricht Lundborg Syndrome" OR "Unverricht-Lundborg Disease" OR "Unverricht-Lundborg Syndrome" OR "unverricht-lundborg" OR "Van Bogaert-Bertrand Syndrome" OR "Vogt-Spielmeyer Disease" OR "Wadia Swami Syndrome" OR "Wadia-Swami Syndrome" OR "Watson Syndrome" OR "Werdnig Hoffman" OR "Werdnig-Hoffmann" OR "Westphall Disease" OR "Westphal-Strumpell" OR "Wilhelmsen-Lynch Disease" OR "Wilson disease" OR "Wilson diseases" OR "wilson syndrome" OR "Wilsons Disease" OR "Wilson's disease" OR "Wolfram Syndrome" OR "X ALD" OR "ziehen oppenheim" OR (Divry AND bogaert) OR (Motor AND "Vocal Tic") OR (Myoencephalopathy AND "lactic acidosis" AND stroke) OR (Schilder AND Addison) OR (Siemerling AND Creutzfeldt) OR "Transthyretin amyloidosis" OR "TTR amyloidosis").ti,ab,kw.)



### Search component #3: Animal population [1]

exp animal experiment/ or exp animal model/ or exp experimental animal/ or exp transgenic animal/ or exp male animal/ or exp female animal/ or exp juvenile animal/ OR animal/ OR chordata/ OR vertebrate/ OR tetrapod/ OR exp fish/ OR amniote/ OR exp amphibia/ OR mammal/ OR exp reptile/ OR exp sauropsid/ OR therian/OR exp monotremate/ OR placental mammals/ OR exp marsupial/ OR Euarchontoglires/ OR exp Afrotheria/ OR exp Boreoeutheria/ OR exp Laurasiatheria/ OR exp Xenarthra/ OR primate/ OR exp Dermoptera/ OR exp Glires/ OR exp Scandentia/ OR Haplorhini/ OR exp prosimian/ OR simian/ OR exp tarsiform/ OR Catarrhini/ OR exp Platyrrhini/ OR ape/ OR exp Cercopithecidae/ OR hominid/ OR exp hylobatidae/ OR exp chimpanzee/ OR exp gorilla/ OR exp orang utan/ OR (animal OR animals OR pisces OR fish OR fishes OR catfish OR catfishes OR sheatfish OR silurus OR arius OR heteropneustes OR clarias OR gariepinus OR fathead minnow OR fathead minnows OR pimephales OR promelas OR cichlidae OR trout OR trouts OR char OR chars OR salvelinus OR salmo OR oncorhynchus OR guppy OR guppies OR millionfish OR poecilia OR goldfish OR goldfishes OR carassius OR auratus OR mullet OR mullets OR mugil OR curema OR shark OR sharks OR cod OR cods OR gadus OR morhua OR carp OR carps OR cyprinus OR carpio OR killifish OR eel OR eels OR anguilla OR zander OR sander OR lucioperca OR stizostedion OR turbot OR turbots OR psetta OR flatfish OR flatfishes OR plaice OR pleuronectes OR platessa OR tilapia OR tilapias OR oreochromis OR sarotherodon OR common sole OR dover sole OR solea OR zebrafish OR zebrafishes OR danio OR rerio OR seabass OR dicentrarchus OR labrax OR morone OR lamprey OR lampreys OR petromyzon OR pumpkinseed OR pumpkinseeds OR lepomis OR gibbosus OR herring OR clupea OR harengus OR amphibia OR amphibian OR amphibians OR anura OR salientia OR frog OR frogs OR rana OR toad OR toads OR bufo OR xenopus OR laevis OR bombina OR epidalea OR calamita OR salamander OR salamanders OR newt OR newts OR triturus OR reptilia OR reptile OR reptiles OR bearded dragon OR pogona OR vitticeps OR iguana OR iguanas OR lizard OR lizards OR anguis fragilis OR turtle OR turtles OR snakes OR snake OR aves OR bird OR birds OR quail OR quails OR coturnix OR bobwhite OR colinus OR virginianus OR poultry OR poultries OR fowl OR fowls OR chicken OR chickens OR gallus OR zebra finch OR taeniopygia OR guttata OR canary OR canaries OR serinus OR canaria OR parakeet OR parakeets OR grasskeet OR parrot OR parrots OR psittacine OR psittacines OR shelduck OR tadorna OR goose OR geese OR branta OR leucopsis OR woodlark OR lullula OR flycatcher OR ficedula OR hypoleuca OR dove OR doves OR geopelia OR cuneata OR duck OR ducks OR greylag OR graylag OR anser OR harrier OR circus pygargus OR red knot OR great knot OR calidris OR canutus OR godwit OR limosa OR lapponica OR meleagris OR gallopavo OR jackdaw OR corvus OR monedula OR ruff OR philomachus OR pugnax OR lapwing OR peewit OR plover OR vanellus OR swan OR cygnus OR columbianus OR bewickii OR gull OR chroicocephalus OR ridibundus OR albifrons OR great tit OR parus OR aythya OR fuligula OR streptopelia OR risoria OR spoonbill OR platalea OR leucorodia OR blackbird OR turdus OR merula OR blue tit OR cyanistes OR pigeon OR pigeons OR columba OR pintail OR anas OR starling OR sturnus OR owl OR athene noctua OR pochard OR ferina OR cockatiel OR nymphicus OR hollandicus OR skylark OR alauda OR tern OR sterna OR teal OR crecca OR oystercatcher OR haematopus OR ostralegus OR shrew OR shrews OR sorex OR araneus OR crocidura OR russula OR european mole OR talpa OR chiroptera OR bat OR bats OR eptesicus OR serotinus OR myotis OR dasycneme OR daubentonii OR pipistrelle OR pipistrellus OR cat OR cats OR felis OR catus OR feline OR dog OR dogs OR canis OR canine OR canines OR otter OR otters OR lutra OR badger OR badgers OR meles OR fitchew OR fitch OR fougart or foulmart OR ferrets OR ferret OR polecat OR polecats OR mustela OR putorius OR weasel OR weasels OR fox OR foxes OR vulpes OR common seal OR phoca OR vitulina OR grey seal OR halichoerus OR horse OR horses OR equus OR equine OR equidae OR donkey OR donkeys OR mule OR mules OR pig OR pigs OR swine OR swines OR hog OR hogs OR boar OR boars OR porcine OR piglet OR piglets OR sus OR scrofa OR llama OR llamas OR lama OR glama OR deer OR deers OR cervus OR elaphus OR cow OR cows OR bos taurus OR bos indicus OR bovine OR bull OR bulls OR cattle OR bison OR bisons OR sheep OR sheeps OR ovis aries OR ovine

OR lamb OR lambs OR mouflon OR mouflons OR goat OR goats OR capra OR caprine OR chamois OR rupicapra OR leporidae OR lagomorpha OR lagomorph OR rabbit OR rabbits OR oryctolagus OR cuniculus OR laprine OR hares OR lepus OR rodentia OR rodent OR rodents OR murinae OR mouse OR mice OR mus OR musculus OR murine OR woodmouse OR apodemus OR rat OR rats OR rattus OR norvegicus OR guinea pig OR guinea pigs OR cavia OR porcellus OR hamster OR hamsters OR mesocricetus OR cricetus OR gerbil OR gerbils OR jird OR jirds OR meriones OR unguiculatus OR jerboa OR jerboas OR jaculus OR chinchilla OR chinchillas OR beaver OR beavers OR castor fiber OR castor canadensis OR sciuridae OR squirrel OR squirrels OR sciurus OR chipmunk OR chipmunks OR marmot OR marmots OR marmota OR suslik OR susliks OR spermophilus OR cynomys OR cottonrat OR cottonrats OR sigmodon OR vole OR voles OR microtus OR myodes OR glareolus OR primate OR primates OR prosimian OR prosimians OR lemur OR lemurs OR lemuridae OR loris OR bush baby OR bush babies OR bushbaby OR bushbabies OR galago OR galagos OR anthropoidea OR anthropoids OR simian OR simians OR monkey OR monkeys OR marmoset OR marmosets OR callithrix OR cebuella OR tamarin OR tamarins OR saguinus OR leontopithecus OR squirrel monkey OR squirrel monkeys OR saimiri OR night monkey OR night monkeys OR owl monkey OR owl monkeys OR douroucoulis OR aotus OR spider monkey OR spider monkeys OR ateles OR baboon OR baboons OR papio OR rhesus monkey OR macaque OR macaca OR mulatta OR cynomolgus OR fascicularis OR green monkey OR green monkeys OR chlorocebus OR vervet OR vervets OR pygerythrus OR hominoidea OR ape OR apes OR hylobatidae OR gibbon OR gibbons OR siamang OR siamangs OR nomascus OR symphalangus OR hominidae OR orangutan OR orangutans OR pongo OR chimpanzee OR chimpanzees OR pan troglodytes OR bonobo OR bonobos OR pan paniscus OR gorilla OR gorillas OR troglodytes).ti,ab.

**Embase Search Strategy: ((#1 AND #2) AND #3))**

## 2. PubMed

### Search component #1': Intervention

((Biological transport [mesh] OR cell-penetrating peptides [mesh] OR drug carriers [mesh] OR drug delivery systems [mesh] OR liposomes [mesh] OR nanoparticles [mesh] OR peptides [mesh:noexp]) OR (AAV [tiab] OR Adeno-associated [tiab] OR Cationic lipid [tiab] OR Cationic lipids [tiab] OR "Cell-penetrating peptide" [tiab] OR "Cell-penetrating peptides" [tiab] OR Chitosan [tiab] OR Conjugate [tiab] OR Conjugated [tiab] OR Conjugates [tiab] OR Conjugation [tiab] OR Copolymer[tiab] OR Co-polymer[tiab] OR Co-polymers[tiab] OR Copolymers[tiab] OR CPP [tiab] OR CPPs [tiab] OR Dendrimer [tiab] OR Dendrimers [tiab] OR Dendriplex [tiab] OR Dendriplexes [tiab] OR Excipient [tiab] OR Excipients [tiab] OR Formulation [tiab] OR Formulations [tiab] OR Lipofectamine [tiab] OR Lipofection [tiab] OR Lipoplex [tiab] OR Lipoplexes [tiab] OR liposome [tiab] OR liposomes [tiab] OR Nanoballs [tiab] OR Nanocapsule [tiab] OR Nanocapsules [tiab] OR Nanocarrier [tiab] OR Nanocarriers [tiab] OR Nanocomposite [tiab] OR Nanocomposites [tiab] OR Nanoconjugate [tiab] OR Nanoconjugates [tiab] OR Nanocrystal [tiab] OR Nanocrystals [tiab] OR nanoparticle [tiab] OR nanoparticles [tiab] OR nanoparticulate [tiab] OR Nanorod [tiab] OR Nanorods [tiab] OR Nanotube [tiab] OR Nanotubes [tiab] OR Nano-balls [tiab] OR Nano-capsule [tiab] OR Nano-capsules [tiab] OR Nano-carrier [tiab] OR Nano-carriers [tiab] OR Nano-composite [tiab] OR Nano-composites [tiab] OR Nano-conjugate [tiab] OR Nano-conjugates [tiab] OR Nano-crystal [tiab] OR Nano-crystals [tiab] OR Nano-particle [tiab] OR Nano-particles [tiab] OR nano-particulate [tiab] OR Nano-rod [tiab] OR Nano-rods [tiab] OR Nano-tube [tiab] OR Nano-tubes [tiab] OR Oligoplex [tiab] OR Oligoplexes [tiab] OR PEI [tiab] OR peptide-linked [tiab] OR PLGA [tiab] OR Polyelectrolyte [tiab] OR Polyelectrolytes [tiab] OR Polyethylenimine [tiab] OR polylactic-co-glycolic acid [tiab] OR Polymersomes [tiab] OR Polyplex [tiab] OR Polyplexes [tiab] OR Protamine [tiab] OR Silica [tiab] OR Thiomers [tiab] OR Thiomers [tiab] OR Vector [tiab] OR Vectorized [tiab] OR Vectors [tiab] OR Viral [tiab] OR Virosome [tiab] OR Virosomes [tiab] OR Virus [tiab] OR Viruses [tiab] OR Virus-like particle [tiab] OR Virus-like particles [tiab] OR VLP [tiab] OR VLPs [tiab])) AND ((Morpholinos [mesh] OR oligonucleotides [mesh:noexp] OR Oligonucleotides, Antisense [mesh] OR Oligoribonucleotides [mesh] OR Peptide Nucleic Acids [mesh] OR Phosphorothioate Oligonucleotides [mesh]) OR (antisense [tiab] OR AON [tiab] OR AONs [tiab] OR ASO [tiab] OR ASOs [tiab] OR LNA [tiab] OR LNAs [tiab] OR Locked nucleic acid [tiab] OR Locked nucleic acids [tiab] OR MORF [tiab] OR MORFs [tiab] OR Morpholino [tiab] OR Morpholinos [tiab] OR Peptide nucleic acid [tiab] OR Peptide nucleic acids [tiab] OR Phosphorothioate [tiab] OR Phosphorothioates [tiab] OR PMO [tiab] OR PMOs [tiab] OR PNA [tiab] OR PNAs [tiab] OR SCO [tiab] OR SCOs [tiab] OR splice-correcting oligonucleotide [tiab] OR splice-correcting oligonucleotides [tiab] OR splice-switching oligonucleotide [tiab] OR splice-switching oligonucleotides [tiab] OR SSO [tiab] OR SSOs [tiab]))

### Search component #2': Disease

((((degenerative [tiab] AND nervous system [tiab]) OR (degenerative [tiab] AND neurologic [tiab]) OR (degenerative [tiab] AND neurological [tiab]) OR (degenerative [tiab] AND spinal cord [tiab]) OR fasciculation [tiab] OR neurodegenerative [tiab] OR neuromuscular [tiab]) AND (autosomal [tiab] OR congenital [tiab] OR dominant [tiab] OR familial [tiab] OR genealogical [tiab] OR genetic [tiab] OR hereditary [tiab] OR heritable [tiab] OR inborn [tiab] OR inheritable [tiab] OR inheritance [tiab] OR inherited [tiab] OR recessive [tiab] OR single-gene [tiab] OR X-linked [tiab]) OR ((Siemerling[tiab] AND Creutzfeldt[tiab]) OR "Adenoma Sebaceum"[tiab] OR "adreno leucodystrophy"[tiab] OR "adreno leukodystrophy"[tiab] OR "adrenoleucodystrophy"[tiab] OR "Adrenoleukodystrophy"[tiab] OR "adrenoleukomyeloneuropathy"[tiab] OR "adrenomyeloneuropathy"[tiab] OR "Alexander Disease"[tiab] OR "alexander syndrome"[tiab] OR "Alexanders Disease"[tiab] OR "Alexander's Disease"[tiab] OR "Alstrom Hallgren Syndrome"[tiab] OR "Alstrom Syndrome"[tiab] OR "Alström Syndrome"[tiab] OR "Alstrom-Hallgren Syndrome"[tiab] OR "Alstrom's Syndrome"[tiab] OR "Alzheimer's"[tiab] OR "Alzheimer"[tiab] OR "Alzheimers"[tiab] OR "amaurotic familial idiocy"[tiab] OR "amaurotic idiocy"[tiab] OR

"amyloid neuropathies"[tiab] OR "Amyloid Neuropathy"[tiab] OR "Amyloid Polyneuropathies"[tiab] OR "amyloid polyneuropathy"[tiab] OR "amyloidotic neuropathy"[tiab] OR "amyloidotic polyneuropathy"[tiab] OR "Amyotrophic Lateral Sclerosis"[tiab] OR "analgesia congenita"[tiab] OR "Analphalipoproteinemia"[tiab] OR "Anterior Horn Cell Disease"[tiab] OR "ARSA Deficiency"[tiab] OR "Arylsulfatase A Deficiencies"[tiab] OR "Arylsulfatase A Deficiency"[tiab] OR "ASP Deficiency"[tiab] OR "ASPA Deficiency"[tiab] OR "Aspartoacylase Deficiency"[tiab] OR "Ataxia Telangiectasia Syndrome"[tiab] OR "Ataxia Telangiectasia"[tiab] OR "ataxia teleangiectasia"[tiab] OR "Autosomal Dominant Optic Atrophy"[tiab] OR "Autosomal Dominant Spastic Paraplegia"[tiab] OR "Autosomal Dominant Striatonigral Degeneration"[tiab] OR "Autosomal Recessive Spastic Paraplegia"[tiab] OR "Azorean Disease"[tiab] OR "balo disease"[tiab] OR "Baltic Myoclonus"[tiab] OR "bar syndrome"[tiab] OR "Batten"[tiab] OR "Becker Disease"[tiab] OR "Becker Muscular Dystrophy"[tiab] OR "Becker's Muscular Dystrophy"[tiab] OR "beta-Galactosidase Deficiencies"[tiab] OR "beta-Galactosidase Deficiency"[tiab] OR "Bilateral Acoustic Neuroma"[tiab] OR "Bilateral Acoustic Neuromas"[tiab] OR "Bilateral Acoustic Schwannoma"[tiab] OR "Bilateral Acoustic Schwannomas"[tiab] OR "bourneville disease"[tiab] OR "Bourneville Phakomatosis"[tiab] OR "bourneville pringle syndrome"[tiab] OR "Bourneville Pringle's Disease"[tiab] OR "bourneville syndrome"[tiab] OR "bourneville tuberous sclerosis"[tiab] OR "Bourneville-Pringle Disease"[tiab] OR "Bourneville-Pringle's Disease"[tiab] OR "Bourneville's Disease"[tiab] OR "Bourneville's Syndrome"[tiab] OR "brain sclerosis"[tiab] OR "bulbar dysfunction"[tiab] OR "bulbar muscle weakness"[tiab] OR "Bulbar palsies"[tiab] OR "Bulbar Palsy"[tiab] OR "bulbar paralysis"[tiab] OR "bulbar sign"[tiab] OR "bulbar symptom"[tiab] OR "bulbar syndrome"[tiab] OR "bulbar weakness"[tiab] OR "Bulbospinal atrophy"[tiab] OR "bulbospinal muscular atrophy"[tiab] OR "bulbo-spinal muscular atrophy"[tiab] OR "Bulbospinal Neuronopathy"[tiab] OR "Canavan Disease"[tiab] OR "canavan syndrome"[tiab] OR "Canavan's disease"[tiab] OR "Canavan-van Bogaert-Bertrand Disease"[tiab] OR "Central core disease"[tiab] OR "Central Core Diseases"[tiab] OR "Central Core Myopathies"[tiab] OR "Central Core Myopathy"[tiab] OR "Cerebellar Degeneration with Slow Eye Movements"[tiab] OR "cerebellar dyssynergia"[tiab] OR "Cerebral Scleroses"[tiab] OR "cerebral sclerosis"[tiab] OR "Cerebroside Sulphatase Deficiency"[tiab] OR "Ceroid Storage Disease"[tiab] OR "Ceroid Storage Diseases"[tiab] OR "Channelopathy-Associated Insensitivity To Pain"[tiab] OR "Charcot Disease"[tiab] OR "charcot marie disease"[tiab] OR "Charcot Marie Tooth"[tiab] OR "Cholesterol Thesaurismosis"[tiab] OR "chronic progressive chorea"[tiab] OR "Chronic Progressive External Ophthalmoplegia"[tiab] OR "Chronic Progressive Hereditary Chorea"[tiab] OR "Circumscribed Lobar Atrophy of the Brain"[tiab] OR "Cockayne Syndrome"[tiab] OR "Cockayne"[tiab] OR "Cockayne's"[tiab] OR "concentric sclerosis"[tiab] OR "congenital analgesia"[tiab] OR "congenital atonic sclerotic muscular dystrophy"[tiab] OR "congenital generalized analgesia"[tiab] OR "congenital indifference to pain"[tiab] OR "congenital insensitivity to pain"[tiab] OR "Congenital Myopathy"[tiab] OR "Congenital Myotonia"[tiab] OR "Congenital Pain Insensitivity"[tiab] OR "congenital paramyotonia"[tiab] OR "Continuous Muscle Activity Syndrome"[tiab] OR "Copper Storage Disease"[tiab] OR "CPEO with Ragged Red Fibers"[tiab] OR "cranial nerve paralysis"[tiab] OR "DDPAC"[tiab] OR "dejerine sottas"[tiab] OR "Dejerine-Thomas"[tiab] OR "dementia with Lewy bodies"[tiab] OR "dementia with Lewy body"[tiab] OR "Dementia with Lobar Atrophy and Neuronal Cytoplasmic Inclusions"[tiab] OR "DIDMOAD"[tiab] OR "Dilated Cardiomyopathy"[tiab] OR "Disinhibition-Dementia-Parkinsonism-Amyotrophy"[tiab] OR "Distal 1 Myopathy"[tiab] OR "Distal Muscular Dystrophies"[tiab] OR "Distal Muscular Dystrophy"[tiab] OR "Distal Myopathies"[tiab] OR "Distal Myopathy"[tiab] OR "Dominant Hereditary Sensory Neuropathy"[tiab] OR "Dominant Optic Atrophies"[tiab] OR "dominant optic atrophy"[tiab] OR "Duchenne"[tiab] OR "dysantonomia"[tiab] OR "Dysmyelinogenic Leukodystrophy"[tiab] OR "Dystonia Musculorum Deformans"[tiab] OR "dystrophia myotonica"[tiab] OR "dystrophic myotonia"[tiab] OR "Emery-Dreifuss"[tiab] OR "encephalitis periaxialis"[tiab] OR "epiloia"[tiab] OR "Eulenburg"[tiab] OR "Faciocapulohumeral"[tiab] OR "Facio-Scapulo-Humeral"[tiab] OR "Faciocapuloperoneal"[tiab] OR "familial autonomic dysfunction"[tiab] OR "Familial dysautonomia"[tiab] OR "familial dysautonomy"[tiab] OR "Familial Fatal Insomnia"[tiab] OR "Familial Periodic Paralysis"[tiab] OR "Familial Periodic Paralysis"[tiab] OR "Familial Polyneuropathies"[tiab] OR "Familial Polyneuropathy"[tiab] OR "familial spastic paralysis"[tiab] OR "familial spastic paraparesis"[tiab] OR "familial spastic paraplegia"[tiab] OR

"Fatal Familial Insomnia"[tiab] OR "Fazio-Londe Disease"[tiab] OR "Fazio-Londe Syndrome"[tiab] OR "Fibrinoid Degeneration of Astrocytes"[tiab] OR "Fibrodysplasia Ossificans"[tiab] OR "Friedreich Ataxia"[tiab] OR "Friedreich"[tiab] OR "Friedreich's"[tiab] OR "Friedreichs"[tiab] OR "frontal dementia"[tiab] OR "frontal lobe dementia"[tiab] OR "Frontotemporal Dementia"[tiab] OR "frontotemporal dementias"[tiab] OR "Frontotemporal Lobar Degeneration"[tiab] OR "Frontotemporal Lobe Dementia"[tiab] OR "FTD"[tiab] OR "FTDP"[tiab] OR "FTLD"[tiab] OR "Fukuhara disease"[tiab] OR "Galactocerebrosidase Deficiency"[tiab] OR "Galactosylceramidase Deficiency"[tiab] OR "galactosylceramide lipidosis"[tiab] OR "GALC Deficiency"[tiab] OR "Gehrig Disease"[tiab] OR "Gehrig's Disease"[tiab] OR "Generalized Myotonia"[tiab] OR "Generalized Myotonias"[tiab] OR "Gerstmann-Straussler"[tiab] OR "Gerstmann-Straussler-Scheinker Disease"[tiab] OR "Giant Axonal Neuropathy"[tiab] OR "globoid cell leucodystrophy"[tiab] OR "Globoid Cell Leukodystrophy"[tiab] OR "Globoid Leukodystrophies"[tiab] OR "Globoid Leukodystrophy"[tiab] OR "Glycogen Storage Disease Type VII"[tiab] OR "Glycogen Storage Disease VII"[tiab] OR "Graefe's Disease"[tiab] OR "Greenfield Disease"[tiab] OR "Greenfield's Disease"[tiab] OR "GSD VII"[tiab] OR "GSS"[tiab] OR "Guam Disease"[tiab] OR "Hallervorden Spatz"[tiab] OR "Hallervorden-Spatz"[tiab] OR "Hauptmann-Thannhauser"[tiab] OR "HDDD1"[tiab] OR "HDDD2"[tiab] OR "hemimegalencephaly"[tiab] OR "hepatocerebral degeneration"[tiab] OR "Hepatolenticular Degeneration"[tiab] OR "hepatolenticular syndrome"[tiab] OR "Hereditary Areflexic Dystasia"[tiab] OR "hereditary chorea"[tiab] OR "Hereditary Dysphasic Disinhibition Dementia"[tiab] OR "hereditary motor and sensory neuropathies"[tiab] OR "Hereditary Motor and Sensory Neuropathy "[tiab] OR "Hereditary Motor And Sensory Neuropathy V"[tiab] OR "Hereditary Motor Neuronopathies"[tiab] OR "Hereditary Motor Neuronopathy"[tiab] OR "hereditary motor sensory neuropathy"[tiab] OR "hereditary myotonia"[tiab] OR "Hereditary Optic Atrophies"[tiab] OR "Hereditary Optic Atrophy"[tiab] OR "hereditary optic nerve atrophy"[tiab] OR "Hereditary Optic Neuroretinopathy"[tiab] OR "hereditary sensorimotor neuropathy"[tiab] OR "Hereditary Sensory and Autonomic Neuropathy"[tiab] OR "hereditary sensory and motor neuropathy"[tiab] OR "Hereditary Sensory Neuropathy"[tiab] OR "hereditary spastic paralysis"[tiab] OR "hereditary spastic paraparesis"[tiab] OR "Hereditary Spastic Paraplegia"[tiab] OR "Hereditary Spastic Paraplegias"[tiab] OR "hereditary spinal ataxia"[tiab] OR "Hereditary Spinal Sclerosis"[tiab] OR "heredopathia atactica polyneuritiformis"[tiab] OR "High Density Lipoprotein Deficiency"[tiab] OR "HMSN Type V"[tiab] OR "HMSN V"[tiab] OR "HMSN"[tiab] OR "HOKPP"[tiab] OR "HSAN III"[tiab] OR "HSAN Type III"[tiab] OR "HSAN3"[tiab] OR "HSN-III"[tiab] OR "Huntington disease"[tiab] OR "Huntington"[tiab] OR "Huntington's"[tiab] OR "Hyperkalemic Periodic Paralysis"[tiab] OR "hypokalaemic periodic paralysis"[tiab] OR "Hypokalemic Periodic Paralysis"[tiab] OR "HYPOKPP"[tiab] OR "hypomyelinating Leukodystrophy"[tiab] OR "HYPOPP"[tiab] OR "Inclusion Body Myopathy"[tiab] OR "Inclusion Body Myositides"[tiab] OR "Inclusion Body Myositis"[tiab] OR "Infantile Muscular Atrophies"[tiab] OR "Infantile Muscular Atrophy"[tiab] OR "Inherited Polyneuropathies"[tiab] OR "Inherited Polyneuropathy"[tiab] OR "Isaac Syndrome"[tiab] OR "Isaacs Mertens Syndrome"[tiab] OR "Isaacs Syndrome"[tiab] OR "Isaacs' Syndrome"[tiab] OR "Isaacs-Mertens Syndrome"[tiab] OR "Jansky-Bielschowsky"[tiab] OR "Joseph Azorean Disease"[tiab] OR "joseph disease"[tiab] OR "juvenile amaurotic family idiocy"[tiab] OR "juvenile amaurotic idiocy"[tiab] OR "Juvenile Cerebroretinal Degeneration"[tiab] OR "Juvenile Muscular Atrophies"[tiab] OR "Juvenile Muscular Atrophy"[tiab] OR "Juvenile Optic Atrophy"[tiab] OR "Juvenile-Onset Neuroaxonal Dystrophy"[tiab] OR "Kearn Syndrome"[tiab] OR "Kearne-Sayre"[tiab] OR "Kearns syndrome"[tiab] OR "Kearns' Syndrome"[tiab] OR "Kearns-Sayre"[tiab] OR "Kearns-Sayer"[tiab] OR "Kearns-Sayre Syndrome"[tiab] OR "Kearns-Sayre"[tiab] OR "Kennedy Disease"[tiab] OR "Kennedy's Syndrome"[tiab] OR "Kennedys Disease"[tiab] OR "Kennedy's Disease"[tiab] OR "Kennedy's syndrome"[tiab] OR "Kjer Optic Atrophy"[tiab] OR "Kjer's Optic Atrophy"[tiab] OR "Kjer-Type Optic Atrophy"[tiab] OR "Krabbe"[tiab] OR "Krabbes"[tiab] OR "Krabbe's"[tiab] OR "Kuf Disease"[tiab] OR "Kufs Disease"[tiab] OR "Kuf's Disease"[tiab] OR "Kugelberg-Welander"[tiab] OR "Lafora Disease"[tiab] OR "Lafora"[tiab] OR "Landouzy-Dejerine"[tiab] OR "Lateral Scleroses"[tiab] OR "Lateral Sclerosis"[tiab] OR "Leber Disease"[tiab] OR "Leber Hereditary Optic Atrophy"[tiab] OR "Leber Hereditary optic neuropathy"[tiab] OR "Leber optic atrophy"[tiab] OR "Leber Optic Neuropathy"[tiab] OR "Leber's Disease"[tiab] OR "Leber's Diseases"[tiab] OR "Leber's Hereditary Optic Atrophy"[tiab] OR "Leber's Hereditary Optic Neuropathy"[tiab] OR "Leber's Optic Atrophy"[tiab] OR "Leber's Optic Neuropathy"[tiab]

OR "Leukodystrophy with Rosenthal Fibers"[tiab] OR "Lewy body dementia"[tiab] OR "Lewy body dementias"[tiab] OR "Lewy Body Disease"[tiab] OR "Lewy body diseases"[tiab] OR "Limb-Girdle"[tiab] OR "Lipofuscin Storage Disease"[tiab] OR "Lobar Atrophy Of Brain"[tiab] OR "Lobar Atrophy of the Brain"[tiab] OR "Luft Disease"[tiab] OR "Luft's Disease"[tiab] OR "lundborg syndrome"[tiab] OR "machado disease"[tiab] OR "Machado-Joseph Disease"[tiab] OR "Machado-Joseph"[tiab] OR "macrencephaly"[tiab] OR "Megaconial Myopathy"[tiab] OR "megalencephalic leukodystrophy"[tiab] OR "megalencephaly"[tiab] OR "melanodermic leukodystrophy"[tiab] OR "MELAS Syndrome"[tiab] OR "MELAS"[tiab] OR "Menzel Type OPCA"[tiab] OR "MERRF Syndrome"[tiab] OR "MERRF"[tiab] OR "metachromatic leucodystrophy"[tiab] OR "metachromatic leucoencephalopathy"[tiab] OR "Metachromatic Leukodystrophies"[tiab] OR "Metachromatic Leukodystrophy"[tiab] OR "metachromatic leucoencephalopathy"[tiab] OR "Mitochondrial Encephalomyopathies"[tiab] OR "Mitochondrial Encephalomyopathy"[tiab] OR "Mitochondrial Myopathies"[tiab] OR "Mitochondrial Myopathy"[tiab] OR "Mitochondrial Ocular Myopathy"[tiab] OR "Mitochondrial-Encephalopathy-Lactic Acidosis-Stroke"[tiab] OR "Molluscum Fibrosum"[tiab] OR "morbus wilson"[tiab] OR "motoneuron disease"[tiab] OR "Motor Neuron Disease"[tiab] OR "Motor Neuron Diseases"[tiab] OR "motor neurone disease"[tiab] OR "motor system disease"[tiab] OR "Motor System Diseases"[tiab] OR "multiple mucosal neuroma"[tiab] OR "multiple neurofibroma"[tiab] OR "Multiple Neurofibromas"[tiab] OR "multiple neuroma"[tiab] OR "Multiple System Atrophies"[tiab] OR "Multiple System Atrophy"[tiab] OR "Multiple System Tauopathy with Presenile Dementia"[tiab] OR "Multisystem Atrophies"[tiab] OR "Multisystem Atrophy"[tiab] OR "Multisystemic Atrophies"[tiab] OR "Multisystemic Atrophy"[tiab] OR "muscle dystrophy"[tiab] OR "Muscle Phosphofructokinase Deficiency"[tiab] OR "muscular dystrophia"[tiab] OR "Muscular Dystrophies"[tiab] OR "Muscular Dystrophy"[tiab] OR "Myelopathic Muscular Atrophy"[tiab] OR "Myoclonic Cerebellar Dyssynergia"[tiab] OR "myoclonic epilepsies"[tiab] OR "myoclonic epilepsy"[tiab] OR "Myoclonus Epilepsies"[tiab] OR "Myoclonus epilepsy"[tiab] OR "myodystrophia"[tiab] OR "Myodystrophies"[tiab] OR "myodystrophy"[tiab] OR "Myokymia"[tiab] OR "Myokymias"[tiab] OR "Myositis Ossificans"[tiab] OR "myotonia atrophica"[tiab] OR "Myotonia Congenita"[tiab] OR "myotonia dystrophica"[tiab] OR "myotonia hereditaria"[tiab] OR "Myotonia Levior"[tiab] OR "myotonic atrophy"[tiab] OR "myotonic dystrophia"[tiab] OR "Myotonic Dystrophies"[tiab] OR "Myotonic Dystrophy"[tiab] OR "myotonic muscular dystrophy"[tiab] OR "NBIA"[tiab] OR "Nemaline Myopathies"[tiab] OR "Nemaline Myopathy"[tiab] OR "Nemaline Rod Disease"[tiab] OR "neurodegeneration with brain iron accumulation"[tiab] OR "neurofibromatoses"[tiab] OR "Neurofibromatosis"[tiab] OR "neurogenic amyotrophy"[tiab] OR "neurogenic atrophy"[tiab] OR "neurogenic muscle atrophy"[tiab] OR "neurogenic muscular atrophy"[tiab] OR "neurogenic osteoarthropathy"[tiab] OR "neurogenic paraosteoarthropathy"[tiab] OR "neuromatosis"[tiab] OR "neuromatosis"[tiab] OR "neuromyotonia"[tiab] OR "Neuronal Ceroid-Lipofuscinoses"[tiab] OR "Neuronal Ceroid-Lipofuscinoses"[tiab] OR "neuronal ceroidosis"[tiab] OR "Neuropathic Amyloidosis"[tiab] OR "Nigrospinodental Degeneration"[tiab] OR "Non fluent Primary Progressive Aphasia"[tiab] OR "Non-fluent Primary Progressive Aphasia"[tiab] OR "Nonfluent progressive aphasia"[tiab] OR "Non-fluent progressive aphasia"[tiab] OR "Normokalemic Periodic Paralysis"[tiab] OR "Ocular Muscular Dystrophy"[tiab] OR "oculocranosomatic disease"[tiab] OR "oculocranosomatic syndrome"[tiab] OR "Oculocranosomatic Syndromes"[tiab] OR "Oculopharyngeal"[tiab] OR "olivocerebellar atrophy"[tiab] OR "Olivopontocerebellar Atrophies"[tiab] OR "Olivopontocerebellar Atrophy"[tiab] OR "Olivopontocerebellar"[tiab] OR "Olivo-Ponto-Cerebellar"[tiab] OR "OPCA with Retinal Degeneration"[tiab] OR "Ophthalmoplegia Plus Syndrome"[tiab] OR "Ophthalmoplegia Plus Syndromes"[tiab] OR "Optic Atrophy 1"[tiab] OR "optic atrophy type 1"[tiab] OR "ossifying fibrodysplasia"[tiab] OR "Ossifying myositis"[tiab] OR "Pantothenate Kinase Associated Neurodegeneration"[tiab] OR "Pantothenate Kinase-Associated Neurodegeneration"[tiab] OR "paralysis agitans"[tiab] OR "paramyotonia congenita"[tiab] OR "Parkinson"[tiab] OR "Parkinson's"[tiab] OR "Pelizaeus Merzbacher"[tiab] OR "Pelizaeus-Merzbacher Disease"[tiab] OR "periodic hypokalaemic paralysis"[tiab] OR "periodic hypokalemic paralysis"[tiab] OR "Periodic Paralysis Hypokalemic"[tiab] OR "Periodic Paralysis- Hypokalemic"[tiab] OR "peroneal atrophy"[tiab] OR "peroneal muscle atrophy"[tiab] OR "Peroneal Muscular Atrophies"[tiab] OR "peroneal muscular atrophy"[tiab] OR "PfkM Deficiency"[tiab] OR "Phytanic Acid Oxidase Deficiency"[tiab] OR "phytanic acid storage disease"[tiab] OR "Pick complex"[tiab] OR "Pick dementia"[tiab] OR "Pick

Disease"[tiab] OR "Pick syndrome"[tiab] OR "Pick's complex"[tiab] OR "Picks Disease"[tiab] OR "Pick's disease"[tiab] OR "Pleoclonal Myopathy"[tiab] OR "PNFA"[tiab] OR "PPA Syndrome"[tiab] OR "PPA Syndromes"[tiab] OR "Presenile Dementia"[tiab] OR "Primary Adhalinopathies"[tiab] OR "Primary Adhalinopathy"[tiab] OR "primary dysautonomia"[tiab] OR "primary dysautonomias"[tiab] OR "Primary Parkinsonism"[tiab] OR "Primary Progressive Nonfluent Aphasia"[tiab] OR "pringle bourneville disease"[tiab] OR "pringle disease"[tiab] OR "pringle syndrome"[tiab] OR "Progeria-Like Syndrome"[tiab] OR "Progeria-Like Syndromes"[tiab] OR "progressive external ophthalmoplegia"[tiab] OR "progressive lenticular degeneration"[tiab] OR "Progressive Muscular Atrophies"[tiab] OR "Progressive Muscular Atrophy"[tiab] OR "Progressive nonfluent aphasia"[tiab] OR "Progressive non-fluent aphasia"[tiab] OR "PROMM"[tiab] OR "Proximal Myotonic Myopathies"[tiab] OR "proximal myotonic myopathy"[tiab] OR "Pseudohypertrophic Muscular Dystrophy"[tiab] OR "Pseudomyotonia"[tiab] OR "Pseudosclerosis"[tiab] OR "Psychosine Lipidosis"[tiab] OR "Quantal Squander"[tiab] OR "Ramsay Hunt "[tiab] OR "Recklinghausen disease"[tiab] OR "Recklinghausens Disease"[tiab] OR "Recklinghausen's Disease"[tiab] OR "Refsum Disease"[tiab] OR "Refsum"[tiab] OR "Refsum's"[tiab] OR "Refsums"[tiab] OR "Ricker"[tiab] OR "Riley-Day Syndrome"[tiab] OR "Rod Myopathies"[tiab] OR "Rod Myopathy"[tiab] OR "Rod-Body Myopathy"[tiab] OR "Roussy Lewy"[tiab] OR "Santavuori-Haltia"[tiab] OR "Sarcoglycanopathies"[tiab] OR "Sarcoglycanopathy"[tiab] OR "SBMA"[tiab] OR "SCA1"[tiab] OR "SCA1s"[tiab] OR "SCA2"[tiab] OR "SCA3"[tiab] OR "SCA4"[tiab] OR "SCA5"[tiab] OR "SCA6"[tiab] OR "SCA7"[tiab] OR "Scapulo-ilio-peroneal"[tiab] OR "Scapuloperoneal"[tiab] OR "Sclerosis Tuberosa"[tiab] OR "sebaceous adenoma"[tiab] OR "Semantic Dementia"[tiab] OR "Senile Dementia"[tiab] OR "Shy Drager syndrome"[tiab] OR "Spastic Paraplegia 2"[tiab] OR "Spastic Paraplegia Type 2"[tiab] OR "Spielmeyer-Sjogren"[tiab] OR "Spielmeyer-Vogt"[tiab] OR "Spinal Amyotrophies"[tiab] OR "Spinal Amyotrophy"[tiab] OR "spinal and bulbar muscular atrophy"[tiab] OR "spinal muscle atrophy"[tiab] OR "Spinal Muscular Atrophy"[tiab] OR "spinobulbar muscular atrophy"[tiab] OR "Spinocerebellar Ataxia"[tiab] OR "Spinocerebellar Ataxias"[tiab] OR "Spinocerebellar Atrophies"[tiab] OR "spinocerebellar atrophy"[tiab] OR "Spinocerebellar Degeneration"[tiab] OR "spinocerebellar degenerations"[tiab] OR "Spongiform Leukodystrophy"[tiab] OR "Spongy Degeneration"[tiab] OR "Spongy Disease "[tiab] OR "Steinert"[tiab] OR "Steinerts"[tiab] OR "Steinert's"[tiab] OR "Struempell Lorrain"[tiab] OR "Struempell"[tiab] OR "Strumpel"[tiab] OR "Strumpell Lorrain"[tiab] OR "Strumpell"[tiab] OR "Strumpell's"[tiab] OR "Strumpel's"[tiab] OR "sulfatide lipidosis"[tiab] OR "sulfatidosis"[tiab] OR "sulphatide lipidosis"[tiab] OR "Syndrome of Continuous Muscle Activity"[tiab] OR "Tangier Disease"[tiab] OR "tarui disease"[tiab] OR "Tarui's Disease"[tiab] OR "TDP 43 pathology"[tiab] OR "TDP 43 proteinopathies"[tiab] OR "TDP-43 Proteinopathy"[tiab] OR "Thomsen Disease"[tiab] OR "Thomsen myotonia"[tiab] OR "Thomsen's Disease"[tiab] OR "Thomsen's myotonia"[tiab] OR "Tibial Muscular Dystrophy"[tiab] OR "Torsion dystonia"[tiab] OR "Torsion Dystonias"[tiab] OR "torsion spasm"[tiab] OR "Tourette Syndrome"[tiab] OR "Tourette"[tiab] OR "Tourette's"[tiab] OR "Tourettes"[tiab] OR "tuberose sclerosis"[tiab] OR "Tuberous Sclerosis"[tiab] OR "Udd Myopathy"[tiab] OR "Unverricht Disease"[tiab] OR "Unverricht Lundborg Syndrome"[tiab] OR "Unverricht-Lundborg Disease"[tiab] OR "Unverricht-Lundborg Syndrome"[tiab] OR "unverricht-lundborg"[tiab] OR "Van Bogaert-Bertrand Syndrome"[tiab] OR "Vogt-Spielmeyer Disease"[tiab] OR "Wadia Swami Syndrome"[tiab] OR "Wadia-Swami Syndrome"[tiab] OR "Watson Syndrome"[tiab] OR "Werdnig Hoffman"[tiab] OR "Werdnig-Hoffmann"[tiab] OR "Westphal Disease"[tiab] OR "Westphal-Strumpell"[tiab] OR "Wilhelmsen-Lynch Disease"[tiab] OR "Wilson disease"[tiab] OR "Wilson diseases"[tiab] OR "wilson syndrome"[tiab] OR "Wilson's Disease"[tiab] OR "Wilson's disease"[tiab] OR "Wolfram Syndrome"[tiab] OR "XALD"[tiab] OR "ziehen oppenheim"[tiab] OR (Divry[tiab] AND bogaert[tiab]) OR (Motor[tiab] AND "Vocal Tic"[tiab]) OR (Myoencephalopathy[tiab] AND "lactic acidosis"[tiab] AND stroke[tiab]) OR (Schilder[tiab] AND Addison[tiab]) OR "Transthyretin amyloidosis"[tiab] OR "TTR amyloidosis"[tiab])) OR (Alzheimer Disease [mesh] OR Ataxia Telangiectasia [mesh] OR Heredodegenerative Disorders, Nervous System [mesh] OR Insomnia, Fatal Familial [mesh] OR Isaacs Syndrome [mesh] OR Lewy Body Disease [mesh] OR Mitochondrial Myopathies [mesh] OR Motor Neuron Disease [mesh] OR Multiple System Atrophy [mesh] OR Muscular Disorders, Atrophic [mesh] OR Myasthenia Gravis [mesh] OR Myasthenic Syndromes, Congenital [mesh] OR Myopathies, Structural, Congenital [mesh] OR Myositis [mesh] OR Myotonic Disorders [mesh] OR Pain Insensitivity,

Congenital [mesh] OR Paralyse, Familial Periodic [mesh] OR Parkinson Disease [mesh] OR Pick disease of the brain [mesh] OR Polyneuropathies [mesh] OR TDP-43 Proteinopathies [mesh]))

### Search component #3': Animal population [2]

("animal experimentation"[MeSH Terms] OR "models, animal"[MeSH Terms] OR "invertebrates"[MeSH Terms] OR "Animals"[Mesh:noexp] OR "animal population groups"[MeSH Terms] OR "chordata"[MeSH Terms:noexp] OR "chordata, nonvertebrate"[MeSH Terms] OR "vertebrates"[MeSH Terms:noexp] OR "amphibians"[MeSH Terms] OR "birds"[MeSH Terms] OR "fishes"[MeSH Terms] OR "reptiles"[MeSH Terms] OR "mammals"[MeSH Terms:noexp] OR "primates"[MeSH Terms:noexp] OR "artiodactyla"[MeSH Terms] OR "carnivora"[MeSH Terms] OR "cetacea"[MeSH Terms] OR "chiroptera"[MeSH Terms] OR "elephants"[MeSH Terms] OR "hyraxes"[MeSH Terms] OR "insectivora"[MeSH Terms] OR "lagomorpha"[MeSH Terms] OR "marsupialia"[MeSH Terms] OR "monotremata"[MeSH Terms] OR "perissodactyla"[MeSH Terms] OR "rodentia"[MeSH Terms] OR "scandentia"[MeSH Terms] OR "sirenia"[MeSH Terms] OR "xenarthra"[MeSH Terms] OR "haplorhini"[MeSH Terms:noexp] OR "strepsirhini"[MeSH Terms] OR "platyrrhini"[MeSH Terms] OR "tarsii"[MeSH Terms] OR "catarrhini"[MeSH Terms:noexp] OR "cercopithecidae"[MeSH Terms] OR "hylobatidae"[MeSH Terms] OR "hominidae"[MeSH Terms:noexp] OR "gorilla gorilla"[MeSH Terms] OR "pan paniscus"[MeSH Terms] OR "pan troglodytes"[MeSH Terms] OR "pongo pygmaeus"[MeSH Terms]) OR ((animals[tiab] OR animal[tiab] OR mice[tiab] OR mus[tiab] OR mouse[tiab] OR murine[tiab] OR woodmouse[tiab] OR rats[tiab] OR rat[tiab] OR murinae[tiab] OR muridae[tiab] OR cottonrat[tiab] OR cottonrats[tiab] OR hamster[tiab] OR hamsters[tiab] OR cricetinae[tiab] OR rodentia[tiab] OR rodent[tiab] OR rodents[tiab] OR pigs[tiab] OR pig[tiab] OR swine[tiab] OR swines[tiab] OR piglets[tiab] OR piglet[tiab] OR boar[tiab] OR boars[tiab] OR "sus scrofa"[tiab] OR ferrets[tiab] OR ferret[tiab] OR polecat[tiab] OR polecats[tiab] OR "mustela putorius"[tiab] OR "guinea pigs"[tiab] OR "guinea pig"[tiab] OR cavia[tiab] OR callithrix[tiab] OR marmoset[tiab] OR marmosets[tiab] OR cebuella[tiab] OR hapale[tiab] OR octodon[tiab] OR chinchilla[tiab] OR chinchillas[tiab] OR gerbillinae[tiab] OR gerbil[tiab] OR gerbils[tiab] OR jird[tiab] OR jirds[tiab] OR merione[tiab] OR meriones[tiab] OR rabbits[tiab] OR rabbit[tiab] OR hares[tiab] OR hare[tiab] OR diptera[tiab] OR flies[tiab] OR fly[tiab] OR dipteral[tiab] OR drosophila[tiab] OR drosophilidae[tiab] OR cats[tiab] OR cat[tiab] OR carus[tiab] OR felis[tiab] OR nematoda[tiab] OR nematode[tiab] OR nematodes[tiab] OR sipunculida[tiab] OR dogs[tiab] OR dog[tiab] OR canine[tiab] OR canines[tiab] OR canis[tiab] OR sheep[tiab] OR sheeps[tiab] OR mouflon[tiab] OR mouflons[tiab] OR ovis[tiab] OR goats[tiab] OR goat[tiab] OR capra[tiab] OR capras[tiab] OR rupicapra[tiab] OR rupicapras[tiab] OR chamois[tiab] OR haplorhini[tiab] OR monkey[tiab] OR monkeys[tiab] OR anthropoidea[tiab] OR anthropoids[tiab] OR saguinus[tiab] OR tamarin[tiab] OR tamarins[tiab] OR leontopithecus[tiab] OR hominidae[tiab] OR ape[tiab] OR apes[tiab] OR "pan paniscus"[tiab] OR bonobo[tiab] OR bonobos[tiab] OR "pan troglodytes"[tiab] OR gibbon[tiab] OR gibbons[tiab] OR siamang[tiab] OR siamangs[tiab] OR nomascus[tiab] OR symphalangus[tiab] OR chimpanzee[tiab] OR chimpanzees[tiab] OR prosimian[tiab] OR prosimians[tiab] OR "bush baby"[tiab] OR bush babies[tiab] OR galagos[tiab] OR galago[tiab] OR pongidae[tiab] OR gorilla[tiab] OR gorillas[tiab] OR "pongo pygmaeus"[tiab] OR orangutan[tiab] OR orangutans[tiab] OR lemur[tiab] OR lemurs[tiab] OR lemuriidae[tiab] OR horse[tiab] OR horses[tiab] OR equus[tiab] OR cow[tiab] OR calf[tiab] OR bull[tiab] OR chicken[tiab] OR chickens[tiab] OR gallus[tiab] OR quail[tiab] OR bird[tiab] OR birds[tiab] OR quails[tiab] OR poultry[tiab] OR poultries[tiab] OR fowl[tiab] OR fowls[tiab] OR reptile[tiab] OR reptilia[tiab] OR reptiles[tiab] OR snakes[tiab] OR snake[tiab] OR lizard[tiab] OR lizards[tiab] OR alligator[tiab] OR alligators[tiab] OR crocodile[tiab] OR crocodiles[tiab] OR turtle[tiab] OR turtles[tiab] OR amphibian[tiab] OR amphibians[tiab] OR amphibia[tiab] OR frog[tiab] OR frogs[tiab] OR bombina[tiab] OR salientia[tiab] OR toad[tiab] OR toads[tiab] OR "epidalea calamita"[tiab] OR salamander[tiab] OR salamanders[tiab] OR eel[tiab] OR eels[tiab] OR fish[tiab] OR fishes[tiab] OR pisces[tiab] OR catfish[tiab] OR catfishes[tiab] OR siluriformes[tiab] OR arius[tiab] OR heteropneustes[tiab] OR sheatfish[tiab] OR perch[tiab] OR perches[tiab] OR percidae[tiab] OR perca[tiab] OR trout[tiab] OR trouts[tiab] OR char[tiab] OR chars[tiab] OR salvelinus[tiab] OR



minnow[Tiab] OR cyprinidae[Tiab] OR carps[Tiab] OR carp[Tiab] OR zebrafish[Tiab] OR zebrafishes[Tiab] OR goldfish[Tiab] OR goldfishes[Tiab] OR guppy[Tiab] OR guppies[Tiab] OR chub[Tiab] OR chubs[Tiab] OR tinca[Tiab] OR barbels[Tiab] OR barbuis[Tiab] OR pimphales[Tiab] OR promelas[Tiab] OR "poecilia reticulata"[Tiab] OR mullet[Tiab] OR mullets[Tiab] OR eel[Tiab] OR eels[Tiab] OR seahorse[Tiab] OR seahorses[Tiab] OR mugil curema[Tiab] OR atlantic cod[Tiab] OR shark[Tiab] OR sharks[Tiab] OR catshark[Tiab] OR anguilla[Tiab] OR salmonid[Tiab] OR salmonids[Tiab] OR whitefish[Tiab] OR whitefishes[Tiab] OR salmon[Tiab] OR salmons[Tiab] OR sole[Tiab] OR solea[Tiab] OR lamprey[Tiab] OR lampreys[Tiab] OR pumpkinseed[Tiab] OR sunfish[Tiab] OR sunfishes[Tiab] OR tilapia[Tiab] OR tilapias[Tiab] OR turbot[Tiab] OR turbot[Tiab] OR flatfish[Tiab] OR flatfishes[Tiab] OR sciuridae[Tiab] OR squirrel[Tiab] OR squirrels[Tiab] OR chipmunk[Tiab] OR chipmunks[Tiab] OR suslik[Tiab] OR susliks[Tiab] OR vole[Tiab] OR voles[Tiab] OR lemming[Tiab] OR lemmings[Tiab] OR muskrat[Tiab] OR muskrats[Tiab] OR lemmus[Tiab] OR otter[Tiab] OR otters[Tiab] OR marten[Tiab] OR martens[Tiab] OR martes[Tiab] OR weasel[Tiab] OR badger[Tiab] OR badgers[Tiab] OR ermine[Tiab] OR mink[Tiab] OR minks[Tiab] OR sable[Tiab] OR sables[Tiab] OR gulo[Tiab] OR gulos[Tiab] OR wolverine[Tiab] OR wolverines[Tiab] OR mustela[Tiab] OR llama[Tiab] OR llamas[Tiab] OR alpaca[Tiab] OR alpacas[Tiab] OR camelid[Tiab] OR camelids[Tiab] OR guanaco[Tiab] OR guanacos[Tiab] OR chiroptera[Tiab] OR chiropteras[Tiab] OR bat[Tiab] OR bats[Tiab] OR fox[Tiab] OR foxes[Tiab] OR iguana[Tiab] OR iguanas[Tiab] OR xenopus laevis[Tiab] OR parakeet[Tiab] OR parakeets[Tiab] OR parrot[Tiab] OR parrots[Tiab] OR donkey[Tiab] OR donkeys[Tiab] OR mule[Tiab] OR mules[Tiab] OR zebra[Tiab] OR zebras[Tiab] OR shrew[Tiab] OR shrews[Tiab] OR bison[Tiab] OR bisons[Tiab] OR buffalo[Tiab] OR buffaloes[Tiab] OR deer[Tiab] OR deers[Tiab] OR bear[Tiab] OR bears[Tiab] OR panda[Tiab] OR pandas[Tiab] OR "wild hog"[Tiab] OR "wild boar"[Tiab] OR fitchew[Tiab] OR fitch[Tiab] OR beaver[Tiab] OR beavers[Tiab] OR jerboa[Tiab] OR jerboas[Tiab] OR capybara[Tiab] OR capybaras[Tiab]) NOT medline[SB])

**PubMed Search Strategy: ((#1' AND #2') AND #3')**

### 3. Web of Science

#### Search component #1": Intervention

TS=(AAV OR Adeno-associated OR Cationic lipid OR Cationic lipids OR Cell-penetrating peptide OR Cell-penetrating peptides OR Chitosan OR Conjugate OR Conjugated OR Conjugates OR Conjugation OR Copolymer OR Co-polymer OR Co-polymers OR Copolymers OR CPP OR CPPs OR Dendrimer OR Dendrimers OR Dendriplex OR Dendriplexes OR Excipient OR Excipients OR Formulation OR Formulations OR Lipid OR Lipids OR Lipofectamine OR Lipofection OR Lipoplex OR Lipoplexes OR liposome OR liposomes OR Nanoballs OR Nanocapsule OR Nanocapsules OR Nanocarrier OR Nanocarriers OR Nanocomposite OR Nanocomposites OR Nanoconjugate OR Nanoconjugates OR Nanocrystal OR Nanocrystals OR nanoparticle OR nanoparticles OR Nano-particulate OR Nanorod OR Nanorods OR Nanotube OR Nanotubes OR Nano-ball OR Nano-balls OR Nano-capsule OR Nano-capsules OR Nano-carrier OR Nano-carriers OR Nano-composite OR Nano-composites OR Nano-conjugate OR Nano-conjugates OR Nano-crystal OR Nano-crystals OR Nano-particle OR Nanoparticles OR Nano-particulate OR Nano-rod OR Nano-rods OR Nano-tube OR Nano-tubes OR Oligoplex OR Oligoplexes OR PEI OR peptide OR peptide-linked OR peptides OR PLGA OR Polyelectrolyte OR Polyelectrolytes OR Polyethylenimine OR polylactic-co-glycolic acid OR Polymer OR Polymers OR Polymersomes OR Polyplex OR Polyplexes OR Protamine OR Silica OR Thiomers OR Thiomers OR Transfection OR Vector OR Vectorized OR Vectors OR Viral OR Virosome OR Virosomes OR Virus OR Viruses OR Virus-like particle OR Virus-like particles OR VLP OR VLPs) AND TS=(antisense OR AON OR AONs OR ASO OR ASOs OR LNA OR LNAs OR Locked nucleic acid OR Locked nucleic acids OR MORF OR MORFs OR Morpholino OR Morpholinos OR Peptide nucleic acid OR Peptide nucleic acids OR Phosphorothioate OR Phosphorothioates OR PMO OR PMOs OR PNA OR PNAs OR SCO OR SCOs OR splice-correcting oligonucleotide OR splice-correcting oligonucleotides OR splice-switching oligonucleotide OR splice-switching oligonucleotides OR SSO OR SSOs)

#### Search component #2": Disease

TS=((degenerative AND nervous system) OR (degenerative AND neurologic) OR (degenerative AND neurological) OR (degenerative AND spinal cord) OR fasciculation OR neurodegenerative OR neuromuscular) AND TS=(autosomal OR congenital OR dominant OR familial OR genealogical OR genetic OR hereditary OR heritable OR inborn OR inheritable OR inheritance OR inherited OR recessive OR single-gene OR X-linked) OR TS=("Charcot Marie Tooth" OR "Childhood Torsion Disease" OR "X ALD" OR (Divry AND bogaert) OR (Myoencephalopathy AND "lactic acidosis" AND stroke) OR (Schilder AND Addison) OR (Siemerling AND Creutzfeldt) OR "Adenoma Sebaceum" OR "adreno leucodystrophy" OR "adreno leukodystrophy" OR "adrenoleucodystrophy" OR "Adrenoleukodystrophy" OR "adrenoleukomyeloneuropathy" OR "adrenomyeloneuropathy" OR "Alexander Disease" OR "alexander syndrome" OR "Alexanders Disease" OR "Alexander's Disease" OR "Alstrom Hallgren Syndrome" OR "Alstrom Syndrome" OR "Alström Syndrome" OR "Alstrom-Hallgren Syndrome" OR "Alstroms Syndrome" OR "Alstrom's Syndrome" OR "Alzheimer's" OR "Alzheimer" OR "Alzheimers" OR "amaurotic familial idiocy" OR "amaurotic idiocy" OR "amyloid neuropathies" OR "Amyloid Neuropathy" OR "Amyloid Polyneuropathies" OR "amyloid polyneuropathy" OR "amyloidotic neuropathy" OR "amyloidotic polyneuropathy" OR "Amyotrophic Lateral Sclerosis" OR "analgesia congenita" OR "Analphalipoproteinemia" OR "Anterior Horn Cell Disease" OR "ARSA Deficiency" OR "Arylsulfatase A Deficiencies" OR "Arylsulfatase A Deficiency" OR "ASP Deficiency" OR "ASPA Deficiency" OR "Aspartoacylase Deficiency" OR "Ataxia Telangiectasia Syndrome" OR "Ataxia Telangiectasia" OR "ataxia telangiectatica" OR "ataxia teleangiectasia" OR "Autosomal Dominant Cerebellar Ataxia, Type II" OR "Autosomal Dominant Optic Atrophy" OR "Autosomal Dominant Spastic Paraplegia" OR "Autosomal Dominant Striatonigral Degeneration" OR "Autosomal Recessive Spastic Paraplegia" OR "Azorean Disease" OR "balo disease" OR "Baltic Myoclonus" OR "bar syndrome" OR "Batten" OR "Becker Disease" OR "Becker Muscular Dystrophy" OR "Becker's Muscular Dystrophy" OR "beta-Galactosidase

Deficiencies" OR "beta-Galactosidase Deficiency" OR "Bilateral Acoustic Neurinoma" OR "Bilateral Acoustic Neurinomas" OR "Bilateral Acoustic Neuroma" OR "Bilateral Acoustic Neuromas" OR "Bilateral Acoustic Schwannoma" OR "Bilateral Acoustic Schwannomas" OR "bourneville disease" OR "Bourneville Phakomatosis" OR "bourneville pringle syndrome" OR "Bourneville Pringle's Disease" OR "bourneville syndrome" OR "bourneville tuberous sclerosis" OR "Bourneville-Pringle Disease" OR "Bourneville-Pringles Disease" OR "Bourneville-Pringle's Disease" OR "Bourneville's Disease" OR "Bourneville's Syndrome" OR "brain sclerosis" OR "bulbar and spinal muscular atrophy" OR "bulbar dysfunction" OR "bulbar muscle weakness" OR "Bulbar palsies" OR "Bulbar Palsy" OR "bulbar paralyses" OR "bulbar paralysis" OR "bulbar sign" OR "bulbar symptom" OR "bulbar syndrome" OR "bulbar weakness" OR "Bulbospinal atrophy" OR "Bulbo-Spinal Atrophy" OR "bulbospinal muscular atrophy" OR "bulbo-spinal muscular atrophy" OR "Bulbospinal Neuronopathy" OR "Canavan Disease" OR "canavan syndrome" OR "Canavan's disease" OR "Canavan-van Bogaert-Bertrand Disease" OR "Central core disease" OR "Central Core Diseases" OR "Central Core Myopathies" OR "Central Core Myopathy" OR "Cerebellar Degeneration with Slow Eye Movements" OR "cerebellar dyssynergia" OR "Cerebral Scleroses" OR "cerebral sclerosis" OR "Cerebroside Sulphatase Deficiency" OR "Ceroid Storage Disease" OR "Ceroid Storage Diseases" OR "Channelopathy-Associated Insensitivity To Pain" OR "Charcot Disease" OR "charcot marie disease" OR "Cholesterol Thesaurismosis" OR "chorea major" OR "chronic progressive chorea" OR "Chronic Progressive External Ophthalmoplegia" OR "Chronic Progressive Hereditary Chorea" OR "Circumscribed Lobar Atrophy of the Brain" OR "Cockayne Syndrome" OR "Cockayne" OR "Cockayne's" OR "concentric sclerosis" OR "congenital analgesia" OR "congenital anesthesia" OR "congenital atonic sclerotic muscular dystrophy" OR "congenital generalized analgesia" OR "congenital indifference to pain" OR "congenital insensitivity to pain" OR "Congenital Myopathy" OR "Congenital Myotonia" OR "Congenital Pain Insensitivity" OR "congenital paramyotonia" OR "Continuous Muscle Activity Syndrome" OR "Copper Storage Disease" OR "CPEO with Ragged Red Fibers" OR "cranial nerve paralysis" OR "DDPAC" OR "dejerine sottas" OR "Dejerine-Thomas" OR "dementia with Lewy bodies" OR "dementia with Lewy body" OR "Dementia with Lobar Atrophy and Neuronal Cytoplasmic Inclusions" OR "DIDMOAD" OR "Dilated Cardiomyopathy" OR "Disinhibition-Dementia-Parkinsonism-Amyotrophy" OR "Distal 1 Myopathy" OR "Distal Hereditary Myopathy" OR "Distal Muscular Dystrophies" OR "Distal Muscular Dystrophy" OR "Distal Myopathies" OR "Distal Myopathy" OR "Dominant Hereditary Sensory Neuropathy" OR "Dominant Optic Atrophies" OR "dominant optic atrophy" OR "Duchenne" OR "dysantonomia" OR "Dysmyelinogenic Leukodystrophy" OR "Dyssynergia Cerebellaris " OR "Dystonia Musculorum Deformans" OR "dystrophia myotonica" OR "dystrophic myotonia" OR "Emery-Dreifuss" OR "encephalitis periaxialis" OR "epiloia" OR "Eulenburg" OR "Facioscapulohumeral" OR "Facio-Scapulo-Humeral" OR "Facioscapuloperoneal" OR "Familial Acoustic Neuromas" OR "familial autonomic dysfunction" OR "Familial dysautonomia" OR "familial dysautonomy" OR "Familial Fatal Insomnia" OR "Familial Periodic Paralyses" OR "Familial Periodic Paralysis" OR "Familial Polyneuropathies" OR "Familial Polyneuropathy" OR "familial spastic paralysis" OR "familial spastic paraparesis" OR "familial spastic paraplegia" OR "Fatal Familial Insomnia" OR "Fazio-Londe Disease" OR "Fazio-Londe Syndrome" OR "Fibrinoid Degeneration of Astrocytes" OR "Fibrodysplasia Ossificans" OR "Friedreich Ataxia" OR "Friedreich" OR "Friedreich's" OR "Friedreichs" OR "frontal dementia" OR "frontal lobe dementia" OR "Frontotemporal Dementia" OR "frontotemporal dementias" OR "Frontotemporal Lobar Degeneration" OR "Frontotemporal Lobe Dementia" OR "FTD" OR "FTDP" OR "FTLD" OR "Fukuhara disease" OR "Galactocerebrosidase Deficiency" OR "Galactosylceramidase Deficiency" OR "galactosylceramide lipidosis" OR "GALC Deficiency" OR "Gehrig Disease" OR "Gehrigs Disease" OR "Gehrig's Disease" OR "Generalized Myotonia" OR "Generalized Myotonias" OR "Gerstmann-Straussler" OR "Gerstmann-Straussler-Scheinker Disease" OR "Giant Axonal Neuropathy" OR "globoid cell dystrophy" OR "globoid cell leukodystrophy" OR "Globoid Cell Leukodystrophy" OR "Globoid Leukodystrophies" OR "Globoid Leukodystrophy" OR "Glycogen Storage Disease Type VII" OR "Glycogen Storage Disease VII" OR "Graefe's Disease" OR "Greenfield Disease" OR "Greenfield's Disease" OR "GSD VII" OR "GSS" OR "Guam Disease" OR "Hallervorden Spatz" OR "Hallervorden-Spatz" OR "Hauptmann-Thannhauser" OR "HDDD1" OR "HDDD2" OR "hemimegalencephaly" OR "hepatocerebral degeneration" OR "Hepatolenticular Degeneration" OR "hepatolenticular syndrome" OR

"Hereditary Areflexic Dystasia" OR "hereditary chorea" OR "Hereditary Dysphasic Disinhibition Dementia" OR "hereditary motor and sensory neuropathies" OR "Hereditary Motor and Sensory Neuropathy " OR "Hereditary Motor And Sensory Neuropathy V" OR "Hereditary Motor Neuronopathies" OR "Hereditary Motor Neuronopathy" OR "hereditary motor sensory neuropathy type IV" OR "hereditary motor sensory neuropathy" OR "hereditary myotonia" OR "Hereditary Optic Atrophies" OR "Hereditary Optic Atrophy" OR "hereditary optic nerve atrophy" OR "Hereditary Optic Neuroretinopathy" OR "hereditary sensorimotor neuropathy" OR "Hereditary Sensory and Autonomic Neuropathy" OR "hereditary sensory and motor neuropathy" OR "Hereditary Sensory Neuropathy" OR "hereditary spastic paralysis" OR "hereditary spastic paraparesis" OR "Hereditary Spastic Paraplegia" OR "Hereditary Spastic Paraplegias" OR "hereditary spinal ataxia" OR "Hereditary Spinal Sclerosis" OR "Hereditary Type I Motor and Sensory Neuropathy" OR "heredopathia atactica polyneuritiformis" OR "High Density Lipoprotein Deficiency" OR "HMSN 5" OR "HMSN Type IV" OR "HMSN Type V" OR "HMSN V" OR "HMSN" OR "HOKPP" OR "HSAN III" OR "HSAN Type III" OR "HSAN3" OR "HSN-III" OR "Huntington disease" OR "Huntington" OR "Huntington's" OR "Hyperkalemic Periodic Paralysis" OR "hypokalaemic periodic paralysis" OR "Hypokalemic Periodic Paralysis" OR "HYPOKPP" OR "hypomyelinating Leukodystrophy" OR "HYPOPP" OR "Inclusion Body Myopathy" OR "Inclusion Body Myositides" OR "Inclusion Body Myositis" OR "Infantile Muscular Atrophies" OR "Infantile Muscular Atrophy" OR "Inherited Polyneuropathies" OR "Inherited Polyneuropathy" OR "Isaac Syndrome" OR "Isaacs Mertens Syndrome" OR "Isaacs Syndrome" OR "Isaacs' Syndrome" OR "Isaacs-Mertens Syndrome" OR "Jansky-Bielschowsky" OR "Joseph Azorean Disease" OR "joseph disease" OR "juvenile amaurotic family idiocy" OR "juvenile amaurotic idiocy" OR "Juvenile Cerebroretinal Degeneration" OR "juvenile chorea" OR "Juvenile Muscular Atrophies" OR "Juvenile Muscular Atrophy" OR "Juvenile Optic Atrophy" OR "Juvenile-Onset Neuroaxonal Dystrophy" OR "Kearn Syndrome" OR "Kearne-Sayre" OR "Kearns syndrome" OR "Kearns' Syndrome" OR "Kearn-Sayre" OR "Kearns-Sayer" OR "Kearns-Sayre Syndrome" OR "Kearns-Sayre" OR "Kennedy Disease" OR "Kennedy Syndrome" OR "Kennedys Disease" OR "Kennedy's Disease" OR "Kennedy's syndrome" OR "Kjer Optic Atrophy" OR "Kjer's Optic Atrophy" OR "Kjer-Type Optic Atrophy" OR "Krabbe" OR "Krabbes" OR "Krabbe's" OR "Kuf Disease" OR "Kufs Disease" OR "Kuf's Disease" OR "Kugelberg-Welander" OR "Lafora Disease" OR "Lafora" OR "Landouzy-Dejerine" OR "Lateral Scleroses" OR "Lateral Sclerosis" OR "Leber Disease" OR "Leber Hereditary Optic Atrophy" OR "Leber Hereditary optic atrophy" OR "Leber Hereditary optic neuropathy" OR "Leber optic atrophy" OR "Leber Optic Neuropathy" OR "Lebers Disease" OR "Leber's Disease" OR "Leber's Diseases" OR "Leber's Hereditary Optic Atrophy" OR "Leber's Hereditary Optic Neuropathy" OR "Leber's Optic Atrophy" OR "Lebers Optic Neuropathy" OR "Leber's Optic Neuropathy" OR "Leukodystrophy with Rosenthal Fibers" OR "Lewy body dementia" OR "Lewy body dementias" OR "Lewy Body Disease" OR "Lewy body diseases" OR "Limb-Girdle" OR "Lipofuscin Storage Disease" OR "Lobar Atrophy Of Brain" OR "Lobar Atrophy of the Brain" OR "Lobe Dementia, Frontotemporal" OR "Luft Disease" OR "Lufts Disease" OR "Luft's Disease" OR "lundborg syndrome" OR "machado disease" OR "Machado-Joseph Disease" OR "Machado-Joseph" OR "macrencephaly" OR "Megaconial Myopathy" OR "megalencephalic leukodystrophy" OR "megalencephaly" OR "melanodermic leukodystrophy" OR "MELAS Syndrome" OR "MELAS" OR "Menzel Type OPCA" OR "MERRF Syndrome" OR "MERRF" OR "metachromatic leucodystrophy" OR "metachromatic leucoencephalopathy" OR "Metachromatic Leukodystrophies" OR "Metachromatic Leukodystrophy" OR "metachromatic leucoencephalopathy" OR "Mitochondrial Encephalomyopathies" OR "Mitochondrial Encephalomyopathy" OR "Mitochondrial Myopathies" OR "Mitochondrial Myopathy" OR "Mitochondrial Ocular Myopathy" OR "Mitochondrial-Encephalopathy-Lactic Acidosis-Stroke" OR "Molluscum Fibrosum" OR "morbus wilson" OR "motoneuron disease" OR "Motor Neuron Disease" OR "Motor Neuron Diseases" OR "motor neurone disease" OR "motor system disease" OR "Motor System Diseases" OR "multiple mucosal neuroma" OR "multiple neurofibroma" OR "Multiple Neurofibromas" OR "multiple neuroma" OR "Multiple System Atrophies" OR "Multiple System Atrophy" OR "Multiple System Tauopathy with Presenile Dementia" OR "Multisystem Atrophies" OR "Multisystem Atrophy" OR "Multisystemic Atrophies" OR "Multisystemic Atrophy" OR "muscle dystrophy" OR "Muscle Phosphofructokinase Deficiency" OR "muscular dystrophia" OR "Muscular Dystrophies" OR "Muscular Dystrophy" OR "Myelopathic Muscular Atrophy" OR "Myoclonic Cerebellar Dyssynergia" OR "myoclonic

epilepsies" OR "myoclonic epilepsy" OR "Myoclonus Epilepsies" OR "Myoclonus epilepsy" OR "myodystrophia" OR "Myodystrophies" OR "myodystrophy" OR "Myokymia" OR "Myokymias" OR "Myositis Ossificans" OR "myotonia atrophica" OR "Myotonia Congenita" OR "myotonia dystrophica" OR "myotonia hereditaria" OR "Myotonia Levior" OR "myotonic atrophy" OR "myotonic dystrophia" OR "Myotonic Dystrophies" OR "Myotonic Dystrophy" OR "myotonic muscle dystrophy" OR "myotonic muscular dystrophy" OR "NBI A" OR "Nemaline Myopathies" OR "Nemaline Myopathy" OR "Nemaline Rod Disease" OR "neurodegeneration with brain iron accumulation" OR "neurofibromatoses" OR "Neurofibromatosis" OR "neurogenic amyotrophy" OR "neurogenic atrophy" OR "neurogenic muscle atrophy" OR "neurogenic muscular atrophy" OR "neurogenic osteoarthropathy" OR "neurogenic paraosteoarthropathy" OR "Neurogenic Scapuloperoneal Amyotrophy" OR "neuroinomatosis" OR "neuromatosis" OR "neuromyotonia" OR "Neuronal Ceroid-Lipofuscinoses" OR "Neuronal Ceroid-Lipofuscinosis" OR "neuronal ceroidosis" OR "Neuropathic Amyloidosis" OR "Nigrospinodentatal Degeneration" OR "Non fluent Primary Progressive Aphasia" OR "Non-fluent Primary Progressive Aphasia" OR "Nonfluent progressive aphasia" OR "Non-fluent progressive aphasia" OR "Normokalemic Periodic Paralysis" OR "Ocular Muscular Dystrophy" OR "oculocraniosomatic disease" OR "oculocraniosomatic syndrome" OR "Oculocraniosomatic Syndromes" OR "Oculopharyngeal" OR "olivocerebellar atrophy" OR "Olivopontocerebellar Atrophies" OR "Olivopontocerebellar Atrophy" OR "Olivopontocerebellar" OR "Olivo-Ponto-Cerebellar" OR "OPCA with Retinal Degeneration" OR "Ophthalmoplegia Plus Syndrome" OR "Ophthalmoplegia Plus Syndromes" OR "Optic Atrophy 1" OR "optic atrophy type 1" OR "ossifying fibrodysplasia" OR "Ossifying myositis" OR "Pantothenate Kinase Associated Neurodegeneration" OR "Pantothenate Kinase-Associated Neurodegeneration" OR "paralysis agitans" OR "paramyotonia congenita" OR "Parkinson" OR "Parkinson's" OR "Pelizaeus Merzbacher" OR "Pelizaeus-Merzbacher Disease" OR "periodic hypokalaemic paralysis" OR "periodic hypokalemic paralysis" OR "Periodic Paralysis Hypokalemic" OR "Periodic Paralysis- Hypokalemic" OR "peroneal atrophy" OR "peroneal muscle atrophy" OR "Peroneal Muscular Atrophies" OR "Peroneal Muscular Atrophy with Pyramidal Features" OR "peroneal muscular atrophy" OR "PfkM Deficiency" OR "Phytanic Acid Oxidase Deficiency" OR "phytanic acid storage disease" OR "Pick complex" OR "Pick dementia" OR "Pick Disease" OR "Pick syndrome" OR "Pick's complex" OR "Picks Disease" OR "Pick's disease" OR "Pleocoal Myopathy" OR "PNFA" OR "Polyneuritic Amyloidosis" OR "PPA Syndrome" OR "PPA Syndromes" OR "Presenile Dementia" OR "Primary Adhalinopathies" OR "Primary Adhalinopathy" OR "primary dysautonomia" OR "primary dysautonomias" OR "Primary Parkinsonism" OR "Primary Progressive Nonfluent Aphasia" OR "Primary Senile Degenerative Dementia" OR "pringle bourmeville disease" OR "pringle disease" OR "pringle syndrome" OR "Progeria-Like Syndrome" OR "Progeria-Like Syndromes" OR "progressive external ophthalmoplegia" OR "progressive lenticular degeneration" OR "Progressive Muscular Atrophies" OR "Progressive Muscular Atrophy" OR "Progressive nonfluent aphasia" OR "Progressive non-fluent aphasia" OR "Progressive Torsion Spasm" OR "PROMM" OR "Proximal Myotonic Myopathies" OR "proximal myotonic myopathy" OR "Pseudohypertrophic Muscular Dystrophy" OR "Pseudomyotonia" OR "Pseudosclerosis" OR "Psychosine Lipidosis" OR "Quantal Squander" OR "Ramsay Hunt " OR "Recklinghausen disease" OR "Recklinghausens Disease" OR "Recklinghausen's Disease" OR "Refsum Disease" OR "Refsum" OR "Refsum's" OR "Refsums" OR "Ricker" OR "Riley-Day Syndrome" OR "Rod Myopathies" OR "Rod Myopathy" OR "Rod-Body Myopathy" OR "Roussy Levy" OR "Santavuori-Haltia" OR "Sarcoglycanopathies" OR "Sarcoglycanopathy" OR "sayre kearns syndrome" OR "SBMA" OR "SCA1" OR "SCA1s" OR "SCA2" OR "SCA3" OR "SCA4" OR "SCA5" OR "SCA6" OR "SCA7" OR "Scapulo-ilio-peroneal" OR "Scapuloperoneal" OR "Sclerosis Tuberosa" OR "sebaceous adenoma" OR "Semantic Dementia" OR "Senile Dementia" OR "Shy Drager syndrome" OR "spastic hereditary paraplegia" OR "Spastic Paraplegia 2" OR "Spastic Paraplegia Type 2" OR "Spielmeyer-Sjogren" OR "Spielmeyer-Vogt" OR "Spinal Amyotrophies" OR "Spinal Amyotrophy" OR "spinal and bulbar muscular atrophy" OR "spinal muscle atrophy" OR "Spinal Muscular Atrophy" OR "spine muscle atrophy" OR "spinobulbar muscular atrophy" OR "Spinocerebellar Ataxia" OR "Spinocerebellar Ataxias" OR "Spinocerebellar Atrophies" OR "spinocerebellar atrophy" OR "Spinocerebellar Degeneration" OR "spinocerebellar degenerations" OR "Spongiform Leukodystrophy" OR "Spongy Degeneration" OR "Spongy Disease" OR "Steinert" OR "Steinerts" OR "Steinert's" OR

"Struempell Lorrain" OR "Struempell" OR "Strumpel" OR "Strumpell Lorrain" OR "Strumpell" OR "Strumpell's" OR "Strumpel's" OR "sulfatide lipidosis" OR "sulfatidosis" OR "sulphatide lipidosis" OR "Syndrome of Continuous Muscle Activity" OR "Tangier Disease" OR "tarui disease" OR "Tarui's Disease" OR "TDP 43 pathology" OR "TDP 43 proteinopathies" OR "TDP-43 Proteinopathy" OR "Thomsen Disease" OR "Thomsen myotonia" OR "Thomsens Disease" OR "Thomsen's Disease" OR "Thomsens myotonia" OR "Thomsen's myotonia" OR "Tibial Muscular Dystrophy" OR "Torsion dystonia" OR "Torsion Dystonias" OR "torsion spasm" OR "Tourette Syndrome" OR "Tourette" OR "Tourette's" OR "Tourettes" OR "tuberose sclerosis" OR "Tuberous Sclerosis" OR "Udd Myopathy" OR "Unverricht Disease" OR "Unverricht Lundborg Syndrome" OR "Unverricht-Lundborg Disease" OR "Unverricht-Lundborg Syndrome" OR "unverricht-lundborg" OR "Van Bogaert-Bertrand Syndrome" OR "Vogt-Spielmeyer Disease" OR "Wadia Swami Syndrome" OR "Wadia-Swami Syndrome" OR "Watson Syndrome" OR "Werdnig Hoffman" OR "Werdnig-Hoffmann" OR "Westphall Disease" OR "Westphal-Strumpell" OR "Wilhelmsen-Lynch Disease" OR "Wilson disease" OR "Wilson diseases" OR "wilson syndrome" OR "Wilson's Disease" OR "Wilson's disease" OR "Wolfram Syndrome" OR "ziehen oppenheim" OR (Motor AND "Vocal Tic") OR "Transthyretin amyloidosis" OR "TTR amyloidosis")

### Search component #3’: Animal population (courtesy of Alice Tillema, Radboudumc)

TS=(animal OR animals OR pisces OR fish OR fishes OR catfish OR catfishes OR sheatfish OR silurus OR arius OR heteropneustes OR clarias OR gariepinus OR fathead minnow OR fathead minnows OR pimphales OR promelas OR cichlidae OR trout OR trouts OR char OR chars OR salvelinus OR salmo OR oncorhynchus OR guppy OR guppies OR millionfish OR poecilia OR goldfish OR goldfishes OR carassius OR auratus OR mullet OR mullets OR mugil OR curema OR shark OR sharks OR cod OR cods OR gadus OR morhua OR carp OR carps OR cyprinus OR carpio OR killifish OR eel OR eels OR anguilla OR zander OR sander OR lucioperca OR stizostedion OR turbot OR turbots OR psetta OR flatfish OR flatfishes OR plaice OR pleuronectes OR platessa OR tilapia OR tilapias OR oreochromis OR sarotherodon OR common sole OR dover sole OR solea OR zebrafish OR zebrafishes OR danio OR rerio OR seabass OR dicentrarchus OR labrax OR morone OR lamprey OR lampreys OR petromyzon OR pumpkinseed OR pumpkinseeds OR lepomis OR gibbosus OR herring OR clupea OR harengus OR amphibia OR amphibian OR amphibians OR anura OR salientia OR frog OR frogs OR rana OR toad OR toads OR bufo OR xenopus OR laevis OR bombina OR epidalea OR calamita OR salamander OR salamanders OR newt OR newts OR triturus OR reptilia OR reptile OR reptiles OR bearded dragon OR pogona OR vitticeps OR iguana OR iguanas OR lizard OR lizards OR anguis fragilis OR turtle OR turtles OR snakes OR snake OR aves OR bird OR birds OR quail OR quails OR coturnix OR bobwhite OR colinus OR virginianus OR poultry OR poultries OR fowl OR fowls OR chicken OR chickens OR gallus OR zebra finch OR taeniopygia OR guttata OR canary OR canaries OR serinus OR canaria OR parakeet OR parakeets OR grasskeet OR parrot OR parrots OR psittacine OR psittacines OR shelduck OR tadorna OR goose OR geese OR branta OR leucopsis OR woodlark OR lullula OR flycatcher OR ficedula OR hypoleuca OR dove OR doves OR geopelia OR cuneata OR duck OR ducks OR greylag OR graylag OR anser OR harrier OR circus pygargus OR red knot OR great knot OR calidris OR canutus OR godwit OR limosa OR lapponica OR meleagris OR gallopavo OR jackdaw OR corvus OR monedula OR ruff OR philomachus OR pugnax OR lapwing OR peewit OR plover OR vanellus OR swan OR cygnus OR columbianus OR bewickii OR gull OR chroicocephalus OR ridibundus OR albifrons OR great tit OR parus OR aythya OR fuligula OR streptopelia OR risoria OR spoonbill OR platalea OR leucorodia OR blackbird OR turdus OR merula OR blue tit OR cyanistes OR pigeon OR pigeons OR columba OR pintail OR anas OR starling OR sturnus OR owl OR athene noctua OR pochard OR ferina OR cockatiel OR nymphius OR hollandicus OR skylark OR alauda OR tem OR sterna OR teal OR crecca OR oystercatcher OR haematopus OR ostralegus OR shrew OR shrews OR sorex OR araneus OR crocidura OR russula OR european mole OR talpa OR chiroptera OR bat OR bats OR eptesicus OR serotinus OR myotis OR dasycneme OR daubentonii OR pipistrelle OR pipistrellus OR cat OR cats OR felis OR catus OR feline OR dog OR dogs OR canis OR canine OR canines OR otter OR otters OR lutra OR badger OR badgers OR meles OR fitchew OR fitch OR foumart OR foulmart OR ferrets OR ferret OR polecat OR polecats OR mustela OR putorius OR weasel OR weasels OR fox OR foxes OR vulpes OR common seal OR phoca OR vitulina OR grey seal OR halichoerus OR horse OR horses OR equus OR equine OR equidae OR

donkey OR donkeys OR mule OR mules OR pig OR pigs OR swine OR swines OR hog OR hogs OR boar OR boars OR porcine OR piglet OR piglets OR sus OR scrofa OR llama OR llamas OR lama OR glama OR deer OR deers OR cervus OR elaphus OR cow OR cows OR bos taurus OR bos indicus OR bovine OR bull OR bulls OR cattle OR bison OR bisons OR sheep OR sheeps OR ovis aries OR ovine OR lamb OR lambs OR mouflon OR mouflons OR goat OR goats OR capra OR caprine OR chamois OR rupicapra OR leporidae OR lagomorpha OR lagomorph OR rabbit OR rabbits OR oryctolagus OR cuniculus OR laprine OR hares OR lepus OR rodentia OR rodent OR rodents OR murinae OR mouse OR mice OR mus OR musculus OR murine OR woodmouse OR apodemus OR rat OR rats OR rattus OR norvegicus OR guinea pig OR guinea pigs OR cavia OR porcellus OR hamster OR hamsters OR mesocricetus OR cricetus OR gerbil OR gerbils OR jird OR jirds OR meriones OR unguiculatus OR jerboa OR jerboas OR jaculus OR chinchilla OR chinchillas OR beaver OR beavers OR castor fiber OR castor canadensis OR sciuridae OR squirrel OR squirrels OR sciurus OR chipmunk OR chipmunks OR marmot OR marmots OR marmota OR suslik OR susliks OR spermophilus OR cynomys OR cottonrat OR cottonrats OR sigmodon OR vole OR voles OR microtus OR myodes OR glareolus OR primate OR primates OR prosimian OR prosimians OR lemur OR lemurs OR lemuridae OR loris OR bush baby OR bush babies OR bushbaby OR bushbabies OR galago OR galagos OR anthropoidea OR anthropoids OR simian OR simians OR monkey OR monkeys OR marmoset OR marmosets OR callithrix OR cebuella OR tamarin OR tamarins OR saguinus OR leontopithecus OR squirrel monkey OR squirrel monkeys OR saimiri OR night monkey OR night monkeys OR owl monkey OR owl monkeys OR douroucoulis OR aotus OR spider monkey OR spider monkeys OR ateles OR baboon OR baboons OR papio OR rhesus monkey OR macaque OR macaca OR mulatta OR cynomolgus OR fascicularis OR green monkey OR green monkeys OR chlorocebus OR vervet OR vervets OR pygerythrus OR hominoidea OR ape OR apes OR hylobatidae OR gibbon OR gibbons OR siamang OR siamangs OR nomascus OR symphalangus OR hominidae OR orangutan OR orangutans OR pongo OR chimpanzee OR chimpanzees OR pan troglodytes OR bonobo OR bonobos OR pan paniscus OR gorilla OR gorillas OR troglodytes)

**Web of Science Search Strategy: ((#1'' AND #2'') AND #3'')**

## References

1. de Vries RBM, Hooijmans CR, Tillema A, Leenaars M, Ritskes-Hoitinga M. Updated version of the Embase search filter for animal studies. *Lab Anim.* 2014;48: 88. doi:10.1177/0023677213494374
2. Hooijmans CR, Tillema A, Leenaars M, Ritskes-Hoitinga M. Enhancing search efficiency by means of a search filter for finding all studies on animal experimentation in PubMed. *Lab Anim.* 2010;44: 170–175. doi:10.1258/la.2010.009117