NPC Enrolment

| Label | Response | Mandatory |
|-----------------------------|---|----------------------------|
| Consent | Yes/No/Not required | Mandatory |
| Reason | Free text | Based on previous response |
| Consent date | dd/mmm/yyyy | Based on previous response |
| Patient Id | Autogenerated | Mandatory |
| Date enrolled | dd/mmm/yyyy | Mandatory |
| Date Data Collection Starts | dd/mmm/yyyy | Expected |
| Type of entry | Prospective/Retrospective | Mandatory |
| Lost to follow up | Yes/No/Unknown | Mandatory |
| Lost to follow up date | dd/mmm/yyyy | Based on previous response |
| NPC subtype | NPC1/NPC2/Unknown | Mandatory |
| Diagnosis age | Autogenerated or Year, Month, Day | Mandatory |
| Diagnosis date | dd/mmm/yyyy | Mandatory |
| Clinical Form | Neonatal rapidly fatal (0 years)/Early infantile neurological (<2 years)/Late infantile neurological (2-6 years)/Juvenile neurological)6-15 years)/Adult neurological (>15years)/Unknown neurological/Visceral symptoms only | Mandatory |
| Deceased | Yes/No/Unknown | Mandatory |
| Date of death | dd/mmm/yyyy | Based on previous response |

NPC Baseline

| Label | Response | Mandatory |
|--|--|---------------------------------------|
| Labei | Biochemical and Ge | · · · · · · · · · · · · · · · · · · · |
| DNIA to otion a posificana od | | - |
| DNA testing performed | Yes/No/Unknown | Mandatory |
| NP-C1 mutation identified? | Yes/No/Unknown | Based on previous response |
| Mutation #1 NP-C1 | Free text | Based on previous response |
| Mutation #2 NP-C1 | Free text | Based on previous response |
| Further NPC1 genetic variants identified ? | Yes/No/Unknown | Based on previous response |
| NP-C2 mutation identified? | Yes/No/Unknown | Based on previous response |
| Mutation #1 NP-C2 | Free text | Based on previous response |
| Mutation #2 NP-C2 | Free text | Based on previous response |
| Further NPC2 genetic variants identified ? | Yes/No/Unknown | Based on previous response |
| Age test performed | Year/Month/Day | Based on previous response |
| DNA testing performed lab: | Lab list | Based on previous response |
| Please specify | Free text | prohibited |
| Have parents been studied | Yes/No/Unknown | Based on previous response |
| Father studied | Yes/No/Unknown | Based on previous response |
| Mother studied | Yes/No/Unknown | prohibited |
| Filipin-test performed ? | Yes/No/Unknown | Mandatory |
| Results of Filipin-test | Classic/Variant/Uncertain/Normal | Based on previous response |
| Date test performed | dd/mmm/yyyy or Unknown | Based on previous response |
| Name of laboratory the test performed | Lab list | Based on previous response |
| Please specify | Free text | Mandatory |
| 7-Ketocholesterol tested | Yes/No/Unknown | Based on previous response |
| 7-Ketocholesterol result | Normal/Borderline/Elevated/Unknow n | Based on previous response |
| 7-Ketocholesterol date | dd/mmm/yyyy or Unknown | Based on previous response |
| 7-Ketocholesterol lab | Lab list | Mandatory |
| Please specify | Free text | Based on previous response |
| Cholestane-triol tested ? | Yes/No/Unknown | Based on previous response |
| Cholestane-triol results | Normal/Borderline/Elevated/Unknow n | Based on previous response |
| Cholestane-triol date | dd/mmm/yyyy or Unknown | Based on previous response |
| Cholestane-triol lab | Lab list | Based on previous response |
| | | |

| Please specify | Free text | Based on previous response |
|--|--|----------------------------|
| Sample stored ? | Yes/No/Unknown | Mandatory |
| Samples (Can select multiple) | Blood/DBS/DNA/Fibroblasts/Plasma | Based on previous response |
| | Family His | tory |
| Family history of "Niemann- | | |
| Pick disease" | Yes/No/Unknown | Mandatory |
| Parents consanguinity | Yes/No/Unknown | Mandatory |
| Father's family country of | European/Asian/African/Arab/Latino- | |
| origin | America/Unknown | expected |
| Mother's family country of origin | Yes/No/Unknown | expected |
| Complication during | | |
| pregnancy and delivery | Yes/No/Unknown | expected |
| Specify complication | Free text | expected |
| | Preterm:<37 weeks/Term:37-42 | CAPOSTOS |
| Gestational age | weeks/Post-term:>42 | Based on previous response |
| o o o o o o o o o o o o o o o o o o o | weeks/Unknown | |
| | Clinical His | tory |
| | Viserca | • |
| Suffered from neonatal (< | Voc/No/Halman | Mandatany |
| 28d) Jaundice | Yes/No/Unknown | Mandatory |
| Neonatal Jaundice period | <=14 days/>14 days/Unknown | Based on previous response |
| Ever have | | |
| Hepatomegaly/Splenomegaly | Yes/No/Unknown | Mandatory |
| in neonatal period | | |
| Showed signs of hepatic | Yes/No/Unknown | Mandatary |
| failure in neonatal period | res/No/Offictiowif | Mandatory |
| Splenomegaly | Yes/No/Unknown | Mandatory |
| Splenomegaly (age (in years) | Year/Month/Day | Based on previous response |
| first observed) | rear/Worth, Day | based on previous response |
| Hepatomegaly | Yes/No/Unknown | Mandatory |
| Hepatomegaly (age (in years) | Year/Month/Day | Based on previous response |
| first observed) | • | |
| Pulmonary dysfunction | Yes/No/Unknown | Based on previous response |
| Pulmonary dysfunction age (in | Year/Month/Day | prohibited |
| years) at first observed | · | <u> </u> |
| 5 1 (0111) | Neurolog | |
| Development delay (Child) | Yes/No/Unknown | Mandatory |
| Development delay age (in | Year/Month/Day | Based on previous response |
| years) first observed (Child) Development delay (Motor) | Vos/No/Unknown | Based on previous response |
| Development delay (Motor) | Yes/No/Unknown | based on previous response |
| (Sitting) | Yes/No/Unknown | Based on previous response |
| Development delay (Motor) | | |
| (Sitting) age (years) first | Number | Based on previous response |
| observed | | |
| Development delay (Motor) | Yes/No/Unknown | Pasad an provious response |
| (Standing) | res/NO/Officiown | Based on previous response |
| Development delay (Motor) | | |
| (Standing) age (years) first | Number | Based on previous response |
| observed | | |
| Development delay (Motor) | Yes/No/Unknown | Based on previous response |
| (Walking) | 1 C3/140/ OTIKITOWIT | bused on previous response |
| Development delay (Motor) | | |
| (Walking) age (years) first | Number | Based on previous response |
| observed | | |
| Development delay (Speech) | Yes/No/Unknown | Based on previous response |
| Development delay severity | Slight/Moderate/Severe/Regression | Based on previous response |
| (Speech) | Single of the delicated severed regression | 2000 0 p. 271000 100p01100 |
| Development delay | Yes/No/Unknown | Based on previous response |
| (Cognitive) | ,, | |
| | | |

| Development delay severity (Cognitive) | Slight/Moderate/Severe/Regression | Based on previous response |
|--|--------------------------------------|----------------------------|
| Development delay (Social) | Yes/No/Unknown | Based on previous response |
| Development delay severity | | |
| (Social) | Slight/Moderate/Severe/Regression | Based on previous response |
| Clumsiness/impaired | Yes/No/Unknown | Mandatory |
| coordination | | |
| Clumsiness/impaired | Voor/Month/Dov | Deced on provious response |
| coordination age (in years) first observed | Year/Month/Day | Based on previous response |
| Ataxia | Yes/No/Unknown | Mandatory |
| | | , |
| Ataxia age (yrs) first observed | Year/Month/Day | Based on previous response |
| Ataxia severity | Mild/Moderate/Severe | Based on previous response |
| Cataplexy | Yes/No/Unknown | Mandatory |
| Cataplexy age (years) first | Year/Month/Day | Based on previous response |
| observed | · | |
| Seizure Seizure age (years) first | Yes/No/Unknown | Mandatory |
| observed | Year/Month/Day | Based on previous response |
| Dysarthria | Yes/No/Unknown | Mandatory |
| Dysarthria age (years) first | | • |
| observed | Year/Month/Day | Based on previous response |
| Dysphagia | Yes/No/Unknown | Mandatory |
| Dysphagia age (years) first | Year/Month/Day | Based on previous response |
| observed | · | |
| Dystonia Dystonia age (years) first | Yes/No/Unknown | Mandatory |
| observed | Year/Month/Day | Based on previous response |
| Abnormal muscle tone | Yes/No/Unknown | Mandatory |
| Abnormal muscle tone age | | , |
| (years) first observed | Year/Month/Day | Based on previous response |
| Specify abnormal muscle tone | Hypertonia/Hypotonia | Based on previous response |
| Abnormal Vertical Saccades | Yes/No/Unknown | Mandatory |
| Abnormal Vertical Saccades | | • |
| age (years) first observed | Year/Month/Day | Based on previous response |
| Specify abnormal vertical | Slow/Absont | Dasad on provious response |
| saccades | Slow/Absent | Based on previous response |
| Vertical gaze palsy | Yes/No/Unknown | Mandatory |
| Vertical gaze palsy age | Year/Month/Day | Based on previous response |
| Hearing impairment | Yes/No/Unknown | Mandatory |
| Hearing Impairment age (years) first observed | Year/Month/Day | Based on previous response |
| (years) first observed | Cognitive and pos | sychiatric |
| Cognitive impairment | Yes/No/Unknown | Mandatory |
| Cognitive impairment age | | · |
| (yrs) first observed | Year/Month/Day | Based on previous response |
| Cognitive impairment type | Attention/Executive/Language/Memo | Based on previous response |
| Psychiatric manifestations | ry Yes/No/Unknown | |
| Pyschiatric manifestations age | | Mandatory |
| (yrs) first observed | Year/Month/Day | Based on previous response |
| (7.57 | Depression/Impulsiveness (blow up of | |
| | temper, tendency towards aggressive | |
| Pyschiatric manifestations | behaviour)/Psychomotor | Based on previous response |
| types | agitation/Psychosis (including | based on previous response |
| | hallucinations, delusions)/Sleeping | |
| | disorder/Other | |
| Please specify | Free text | prohibited |
| Other cognitive and | Yes/No/Unknown | Based on previous response |
| pyschiatric symptoms ? | | <u> </u> |

| Specify other cognitive and pyschiatric symptoms | Free text | Based on previous response |
|--|--|---------------------------------------|
| pyscinatric symptoms | Other clinical signs a | nd symptoms |
| Any other medical illness ? | Yes/No/Unknown | Mandatory |
| Specify other medical illness | Free text | Based on previous response |
| , | Treatmer | |
| | Antacids/Antidepressant/Antiepileptic | |
| Symptomatic thoranics | /Antipsychotics/Antireflux/Antispastic | ovnoctod |
| Symptomatic therapies | /Dopaminergic/Mood | expected |
| | stabilizer/Other/Unknown | |
| Please specify | Free text | Based on previous response |
| Name | Miglustat/Other | Mandatory |
| Please specify | Free text | Mandatory |
| Start date | dd/mmm/yyyy | Based on previous response |
| Ongoing | Yes or No | Mandatory |
| End date | dd/mmm/yyyy 50/75/100/200/500/Other | Mandatory Based on previous response |
| Dose (mg) Please specify | Free text | Mandatory |
| riease specify | Once a day/Twice a day/Three times a | ivialidatoly |
| Frequency | day | Based on previous response |
| Has an adverse event occured | , | |
| to patient | Yes or No | Based on previous response |
| • | D: 1 // | |
| Adverse event | Diarrhoea/Low | Based on previous response |
| | platelets/Tremour/Weight loss/Other | |
| Please specify | Free text | Expected |
| Clinical Trial Medication - | Free text | Mandatory |
| Name | | <u> </u> |
| Start date | dd/mmm/yyyy | Mandatory |
| Ongoing | Yes or No | Based on previous response |
| End date | dd/mmm/yyyy | Mandatory |
| Dose | Free text | Mandatory |
| Frequency | Free text | Based on previous response |
| Has an adverse event occured to patient | Yes or No | Mandatory |
| Adverse event | Free text | Expected |
| Adverse event | Disability S | • |
| | Normal (0)/Clumsiness | |
| | (1)/Autonomus ataxic gait | |
| Ambulation | (2)/Outdoor assisted ambulation | Mandatory |
| | (3)/Indoor assisted ambuation | |
| | (4)/Wheel-chair bound (5) | |
| | Normal (0)/ Tremor (1)/Slight | |
| | dysmetria/dystonia (allows for | |
| | autonomous manipulation) (2)/Mild | |
| Manipulation | dysmetria/dystonia (requires help for | Mandatory |
| | several tasks but is able to feed self) | , |
| | (3)/ Severe dysmetria/dystonia | |
| | (requires assistance in all activities) | |
| | | |
| | (4) | |
| | Normal (0)/ Delayed acquisition | |
| | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) | |
| Sneach | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only | Mandatory |
| Speech | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family | Mandatory |
| Speech | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal | Mandatory |
| Speech | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of | Mandatory |
| Speech | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) | Mandatory |
| | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ | |
| Speech | Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) | Mandatory Mandatory |

| Eye movements | Normal (0)/ Slow ocular pursuit (1)/ Vertical opthalmoplegia (2)/ Complete opthalmoplegia (3) | Mandatory |
|------------------------------|---|----------------------------|
| Seizure | No (0)/ Occasional seizures (1)/ Seizures with AED (2)/ Seizures resistant to AED (3) | Mandatory |
| Total | autocalculated | Expected |
| | Additional Inves | stigation |
| Neuroimaging assessment done | Yes/No/Unknown | Mandatory |
| MRI result | Normal/Abnormal/Unknown/Not done | Based on previous response |
| Date of assessment (MRI) | dd/mmm/yyyy | Based on previous response |
| MRI assessment finding | Cerebellar atrophy/Cortical atrophy/Ventrical dilation/White matter changes/Other | Based on previous response |
| Please specify | Free text | Based on previous response |
| CT result | Yes/No/Unknown | Mandatory |
| Date of assessment (CT) | dd/mmm/yyyy | Based on previous response |
| CT assessment finding | Cerebellar atrophy/Cortical atrophy/Ventrical dilation/White matter changes/Other | Based on previous response |
| Please specify | Free text | Based on previous response |
| Oxysterols | Yes/No/Unknown | prohibited |
| Oxysterols details | Cholestane-3b,5a,6b-triol/7- ketocholesterol | prohibited |

NPC Encounter

| Label | Response | Mandatory | |
|---|-----------------------------------|----------------------------|--|
| Visit date | dd/mmm/yyyy or Unknown | expected | |
| Height (cm) | Number | expected | |
| Height Percentile (%) | Number | expected | |
| Weight (kg) | Number | expected | |
| Weight percentile (%) | Number | expected | |
| вмі | autocalculated | Based on previous response | |
| Significant change since last seen | Yes/No/Unknown | prohibited | |
| Specify change | Improvement/Stable/Deteriorated | prohibited | |
| | Viscera | I | |
| Splenomegaly | Yes/No/Unknown | Mandatory | |
| Hepatomegaly | Yes/No/Unknown | Mandatory | |
| Pulmonary dysfunction | Yes/No/Unknown | Mandatory | |
| Jaundice | Yes/No/Unknown | Mandatory | |
| Neurological | | | |
| Development delay (Child) | Yes/No/Unknown | Mandatory | |
| Development delay (Motor) | Yes/No/Unknown | Based on previous response | |
| Development delay (Motor) (Sitting) | Yes/No/Unknown | Based on previous response | |
| Development delay (Motor) (Standing) | Yes/No/Unknown | Based on previous response | |
| Development delay (Motor) (Walking) | Yes/No/Unknown | Based on previous response | |
| Development delay (Speech) | Yes/No/Unknown | Based on previous response | |
| Development delay severity (Speech) | Slight/Moderate/Severe/Regression | Based on previous response | |
| Development delay (Cognitive) | Yes/No/Unknown | Based on previous response | |
| Development delay severity (Cognitive) | Slight/Moderate/Severe/Regression | Based on previous response | |
| Development delay (Social) | Yes/No/Unknown | Based on previous response | |
| Development delay severity (Social) | Slight/Moderate/Severe/Regression | Based on previous response | |

| | ı | |
|------------------------------|---|----------------------------|
| Clumsiness/impaired | Yes/No/Unknown | Mandatory |
| coordination | | , |
| Ataxia | Yes/No/Unknown | Mandatory |
| Ataxia severity | Mild/Moderate/Severe | Based on previous response |
| Cataplexy | Yes/No/Unknown | Mandatory |
| Seizure | Yes/No/Unknown | Mandatory |
| Dysarthria | Yes/No/Unknown | Mandatory |
| Dysphagia | Yes/No/Unknown | Mandatory |
| Dystonia | Yes/No/Unknown | Mandatory |
| Abnormal muscle tone | Yes/No/Unknown | Mandatory |
| specify abnormal muscle tone | | Based on previous response |
| Vertical gaze palsy | Yes/No/Unknown | Mandatory |
| Hearing impairment | Yes/No/Unknown | Mandatory |
| Abnormal Saccades | Yes/No/Unknown | Based on previous response |
| Specify abnormal saccades | Horizontal/Vertical/Complete | Expected |
| Specify abrioritial saccades | Cognitive and Ps | • |
| Cognitive impairment | | |
| Cognitive impairment | Yes/No/Unknown | Mandatory |
| Cognitive impairment type | Attention/Executive/Language/Memo ry | Based on previous response |
| Psychiatric manifestations | Yes/No/Unknown | Mandatory |
| | Depression/Impulsiveness (blow up of | |
| | temper, tendency towards aggressive | |
| Pyschiatric manifestations | behaviour)/Psychomotor | Docod on municipus manages |
| types | agitation/Psychosis (including | Based on previous response |
| ,, | hallucinations, delusions)/Sleeping | |
| | disorder/Other | |
| Please specify | Free text | Mandatory |
| Other developmental delay | Yes/No/Unknown | |
| | res/No/Offknown | Based on previous response |
| Specify other developmental | Free text | prohibited |
| delay | D: 1:1:: 0 | |
| | Disability S | cale |
| | Normal (0)/Clumsiness | |
| | (1)/Autonomus ataxic gait | |
| Ambulation | (2)/Outdoor assisted ambulation | Mandatory |
| | (3)/Indoor assisted ambuation | |
| | (4)/Wheel-chair bound (5) | |
| | Normal (0)/ Tremor (1)/Slight | |
| | dysmetria/dystonia (allows for | |
| | autonomous manipulation) (2)/Mild | |
| | dysmetria/dystonia (requires help for | |
| Manipulation | several tasks but is able to feed self) | Mandatory |
| | (3)/ Severe dysmetria/dystonia | |
| | | |
| | | |
| | (requires assistance in all activities) | |
| | (4) | |
| | (4) Normal (0)/ Delayed acquisition | |
| | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) | |
| | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only | |
| Speech | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family | Mandatory |
| Speech | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal | Mandatory |
| Speech | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family | Mandatory |
| Speech | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal | Mandatory |
| Speech | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of | Mandatory |
| | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ | , |
| Speech | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily | Mandatory |
| | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric | , |
| | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric button feeding (4) | |
| Swallowing | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric button feeding (4) Normal (0)/ Slow ocular pursuit (1)/ | Mandatory |
| | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric button feeding (4) Normal (0)/ Slow ocular pursuit (1)/ Vertical opthalmoplegia (2)/ | |
| Swallowing | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric button feeding (4) Normal (0)/ Slow ocular pursuit (1)/ Vertical opthalmoplegia (2)/ Complete opthalmoplegia (3) | Mandatory |
| Swallowing Eye movements | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric button feeding (4) Normal (0)/ Slow ocular pursuit (1)/ Vertical opthalmoplegia (2)/ Complete opthalmoplegia (3) No (0)/ Occasional seizures (1)/ | Mandatory Mandatory |
| Swallowing | (4) Normal (0)/ Delayed acquisition (1)/Mild dysarthria (understandable) (2)/ Severe dysarthria (only comprehendable to some family members (3)/ Non-verbal communication (4)/ Absence of communications (5) Normal (0)/ Abnormal chewing (1)/ Occasional dysphagia (2)/ Daily dysphagia (3)/ Nasogastric/gastric button feeding (4) Normal (0)/ Slow ocular pursuit (1)/ Vertical opthalmoplegia (2)/ Complete opthalmoplegia (3) | Mandatory |

| Any other medical illness? Yes/No/Unknown Any other medical illness Free text Antacids/Antidepressant/Antiepleptic Antacids/Antimynyy Antacids/Antidepressant/Antiepleptic Antacids/Antinemynyy Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptic Antacids/Antidepressant/Antiepleptical Antacids/Antidepressant/Antiepleptic Antacids/Antidepress | Total | autocalculated | Expected |
|--|------------------------------|--|---------------------------------------|
| Any other medical illness 7 Specify other medical illness 7 Symptomatic therapies Antacids/Antidepressant/Anticeplileptic / Antipsychotics/Antireflux/Antispastic / Dopaminergic/Mood stabilizer/Other/Unknown Please specify | | l l | · · · · · · · · · · · · · · · · · · · |
| Specify other medical illness Free text Based on previous response | Any other medical illness? | | |
| Symptomatic therapies | | | · · · · · · · · · · · · · · · · · · · |
| Symptomatic therapies / Antipsychotics/Antireflux/Antispastic / Dopaminergic/Mood stabilizer/Other/Luknown Please specify Free text Based on previous response Clinical Trial Medication - Free text Mandatory Start date dd/mmm/yyy Mandatory Ongoing Yes or No Based on previous response End date dd/mmm/yyy Mandatory Dose Free text Mandatory Trequency Frequency Free text Mandatory Trequency Free text Passed on previous response to patient Adverse event occured Yes or No Based on previous response Adverse event occured Yes or No Based on previous response Mandatory Passed Mandatory Disease Modifying Therapies - Miglustat/Other Mandatory Start date dd/mmm/yyy Based on previous response Ongoing Yes or No Mandatory Start date dd/mmm/yyy Based on previous response Ongoing Yes or No Mandatory End date dd/mmm/yyy Mandatory Dose S07/5/100/200/500/Other expected Please specify Free text Based on previous response Frequency Once a day/Twice a day/Three times a day Has an adverse event occured to patient Adverse event Diarrhoea/Low platelets/Tremour/Weight loss/Other Please specify Free text Expected Mandatory Mandatory Please specify Free text Based on previous response Adverse event Diarrhoea/Low platelets/Tremour/Weight loss/Other Please specify Free text Expected MRI result Yes/No/Unknown Based on previous response MRI assessment (finding Ad/mmm/yyy Based on previous response Tree text Based on previous response MRI assessment (finding Ad/mmm/yyy Based on previo | | Treatmer | nts |
| Symptomatic therapies /Dopaminergic/Mood stabilizer/Other/Unknown | | Antacids/Antidepressant/Antiepileptic | |
| Dispaminergic/Mood stabilizer/Other/Unknown Based on previous response | Compando apodio de osposico | /Antipsychotics/Antireflux/Antispastic | avec at a d |
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| Cerebellar atrophy/Cortical atrophy/Ventrical dilation/White Based on previous response mater changes/Other Please specify Free text Based on previous response Oxysterols Yes/No/Unknown Based on previous response Cholestane-3b,5a,6b-triol/7- Based on previous response | | , , | · · · · · · · · · · · · · · · · · · · |
| CT assessment finding atrophy/Ventrical dilation/White Based on previous response mater changes/Other Please specify Free text Based on previous response Oxysterols Yes/No/Unknown Based on previous response Cholestane-3b,5a,6b-triol/7- Based on previous response | | | 1 12 22 |
| mater changes/Other Please specify Free text Based on previous response Oxysterols Yes/No/Unknown Based on previous response Cholestane-3b,5a,6b-triol/7- Rased on previous response | CT assessment finding | · · · | Based on previous response |
| Please specify Free text Based on previous response Oxysterols Yes/No/Unknown Based on previous response Cholestane-3b,5a,6b-triol/7- Based on previous response | | | , |
| Oxysterols Yes/No/Unknown Based on previous response Cholestane-3b,5a,6b-triol/7- Based on previous response | Please specify | | Based on previous response |
| Oxysterols details Cholestane-3b,5a,6b-triol/7- Based on previous response | | | |
| L CONVITEROIS DETAILS L HASED ON DEVIOUS RESPONSE | , | | · · |
| | Oxysterois details | I | Based on previous response |

NPC Medical History and Comorbidites

| Data Point | Response Options | Answer required |
|---------------|------------------|-----------------|
| Date Reported | Date | Required |

| Does the patient have any | | |
|---|---|--|
| non-Niemann Pick disease | Check Box | Required |
| related medical history and/or | | Required |
| co-morbidities? | | |
| Description | Free Text | Dependent on previous response |
| Description ICD-10 Code | ICD-10 List | Dependent on previous response |
| Start Date | Date | Dependent on previous response |
| Ongoing? | Check Box | Dependent on previous response |
| End date | Date | Dependent on previous response |
| Do you suspect a relationship | | |
| to any of the medications the | Check Box | Dependent on previous response |
| patient is currently taking? | CHECK BOX | Dependent on previous response |
| patient is currently taking: | | |
| Do you suspect a relationship | Not Related / Unlikely Related / | |
| to Nieman-Pick Specific | Possibly Related / Definitely Related / | Dependent on previous response |
| Medication? | Not Applicable | |
| Please complete an Adverse | | |
| Drug Reaction form for this | | |
| Nieman-Pick medication | Check Box | Dependent on previous response |
| related medical | | |
| history/comorbidity event | | |
| | | |
| | NPC Medication | ons |
| Data Point | Response Options | Answer required |
| Date Reported | Date | Required |
| Is the patient receiving any | Chark Day | Doguirod |
| medication? | Check Box | Required |
| Medication name | Free Text | Dependent on previous response |
| Indication description | Free Text | Dependent on previous response |
| | Nieuwana Bielo Biereau ICD 40 ander / | |
| ICD-10 Code selection options | Niemann-Pick Disease ICD-10 codes / | Dependent on previous response |
| | | Dependent on previous response |
| | Other Disease Indication ICD-10 codes | Dependent on previous response |
| Niemann Pick Disease ICD-10 | | |
| | E75.240 type A / E75.241 type B | Dependent on previous response |
| Niemann Pick Disease ICD-10 | E75.240 type A / E75.241 type B | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes | | |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- | E75.240 type A / E75.241 type B | Dependent on previous response Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date | E75.240 type A / E75.241 type B ICD-10 List Date | Dependent on previous response Dependent on previous response Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code | E75.240 type A / E75.241 type B ICD-10 List | Dependent on previous response Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Drop-Down List | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Drop-Down List | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Drop-Down List Free Text Check Box | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Check Box Check Box | Dependent on previous response Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Drop-Down List Free Text Check Box Adverse Drug Reaction / Lack of Efficacy / Patient Choice / | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Unaffordability / Clinician Decision / | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? Reason for treatment discontinuation | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Drop-Down List Free Text Check Box Adverse Drug Reaction / Lack of Efficacy / Patient Choice / | Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? Reason for treatment discontinuation Adverse Drug Reaction ID | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Check Box Adverse Drug Reaction / Lack of Efficacy / Patient Choice / Unaffordability / Clinician Decision / Change in Treatment Goals / Other | Dependent on previous response Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? Reason for treatment discontinuation Adverse Drug Reaction ID linked to treatment | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Unaffordability / Clinician Decision / | Dependent on previous response Dependent on previous response |
| Niemann Pick Disease ICD-10 codes Other Disease Indication ICD- 10 Code Start Date Ongoing? End date Dose Unit Please specify Route Please specify Frequency Please specify Was treatment discontinued? Reason for treatment discontinuation Adverse Drug Reaction ID | E75.240 type A / E75.241 type B ICD-10 List Date Check Box Date Numerical Drop-Down List Free Text Drop-Down List Free Text Check Box Adverse Drug Reaction / Lack of Efficacy / Patient Choice / Unaffordability / Clinician Decision / Change in Treatment Goals / Other | Dependent on previous response Dependent on previous response |

NPC Adverse Drug Reactions

| Data Point | Response Options | Answer required |
|---------------------|------------------|-----------------|
| Date reported | Date | Required |
| Has an Adverse Drug | Check Box | Required |
| Reaction occurred? | CHECK BOX | Required |

| Adverse Drug Reaction | | |
|---|---|--------------------------------|
| Description | Free Text | Dependent on previous response |
| Start Date | Date | Dependent on previous response |
| Ongoing? | Check Box | Dependent on previous response |
| End Date | Date | Dependent on previous response |
| Severity | Mild / Moderate / Severe | Dependent on previous response |
| Do you suspect a relationship to any of the medications the patient is currently taking? | | Dependent on previous response |
| Do you suspect a relationship to Nieman Pick Specific Medication? | Not Related / Unlikely Related / Possibly Related / Probably Related / Defintiely Related / Not Applicable | Dependent on previous response |
| Seriousness | Not Serious / Death / Life-Threatening / Requires Inpatient Hospitalisation or Prolongation of Existing Hospitalisation / Resulted in Persistent or Significant Incapacity or Disability / Congential Abnormality or Birth Defect / Other Medically Important Event | Dependent on previous response |
| Specify other medically | Free Text | Dependent on previous response |
| important event Initial hospitalisation or Prolongation of current hospitalisation episode? | Initial / Prolongation | Dependent on previous response |
| Admission date | Date | Dependent on previous response |
| Discharge date | Date | Dependent on previous response |
| Outcome | Recovered/Resolved / Recovering/Resolving / Not Recovered/Not Resolved / Recovered/Resolved with Sequelae / Fatal / Unknown | Dependent on previous response |
| Has any medication been discontinued as a result of ADR ? | Yes / No / Unknown | Dependent on previous response |
| Did the ADR abate after dose interruption/discontinuation? | Yes / No / Unknown | Dependent on previous response |
| | NPC Pregna | ancv |
| Data Point | Response Options | Answer required |
| Encounter Date | Date | Required |
| Has Pregnancy occurred? | Yes / No / Unknown | Required |
| Date Reported | Date | Dependent on previous response |
| Date of Last Menstrual Period | | Dependent on previous response |
| Outcome of Pregnancy | Live Birth / Fetal Death / Termination / Spontaneous Abortion | Dependent on previous response |
| Health Status of live birth | Free Text | Dependent on previous response |
| | | |
| NPC End of Data Collection | | |
| Data Point | Response Options | Answer required |
| End of participation date Reason for withdrawl of | Date | Required |
| consent | Deceased / Lost to Follow Up / Other | Dependent on previous response |
| Please specify | Free Text | Dependent on previous response |
| Date of Death | Date | Dependent on previous response |
| Lost to follow up date | Date | Dependent on previous response |
| Status at end of study | Alive / Deceased / Unknown | Dependent on previous response |
| Cause of Death | Free Text | Dependent on previous response |