SURVEY for patients suffering from Canavan Disease

Date when this questionnaire was completed / /
day month year

Patient's name: ____________________
(First) (Last)

Gender: ▢ **M** ▢ **F**

Date of birth of your child / /
day month year

If deceased, date of death / /
day month year

Name of physician primarily responsible for your child's care ____________________

Medical center where your child received care____________________

Name of person completing this form: ____________________

Possibility of contact (e-mail/ phone): ____________________

Dear Family,

Please fill out this form to the best of your knowledge and send it back to us within 2 weeks. Leave out answers that you are not sure about. You can always contact us for support:

Dr. Annette Bley
University Hospital Eppendorf
Pediatric Neurology
Hamburg, Germany
Phone 0049-40-74105-6391
Email: abley@uke.de

We also may contact you to clarify some of the answers.

Family history

In which country/city does your child live (country of origin)? ____________________________

Are the biologic parents related to each other by blood? ▢ **yes** ▢ **no** ▢ **unknown**

Does the child have **relatives that are / have been affected** by the same disease? ▢**yes** ▢ **no**

If other family members are affected:

How many siblings are affected? _______

How many siblings are unaffected? _______

How many other relatives are affected? _______

If your child has **other relatives** that are affected by the same disease, **how are they related to your child**? ____

(i.e. maternal uncle of the child)

What are the **ethnic backgrounds** of the biologic parents? (Hispanic or non hispanic)

Mother ____________________________ **Father** __________________________________

What is the race of the biological parents?

Mother ____________________________ **Father** __________________________________

Pregnancy and Perinatal history

Did the mother of the child **take any drugs during pregnancy**?

▢**yes** ▢**no** ▢ **unknown**

Was **delivery at full term (after 38th week of pregnancy)**? ▢**yes** ▢**no**

If not, at what gestational age was your child born? _____ weeks

Birth weight _________ lbs / grams

Length at birth _________ inches / cm

APGAR score ___ / ___ / ___ (1 min / 5 min / 10 min)

Was the **baby healthy after birth**? ▢**yes** ▢**no** ▢ **unknown**

If the baby was not healthy after birth, **problems in the neonatal period** were due to:

(please check all that apply)

▢ **Respiratory distress**
▢ **Apnea** (did not breath)
▢ **Stridor** (high-pitched wheezing sound)
▢ **Feeding problems**
▢ **Icterus** (jaundice)
▢ **Infections**
▢ **Other** (please specify: __________________)
▢ **Metabolic abnormalities**
▢ **Neurological abnormalities**
▢ **Head ultrasound abnormalities**
▢ **Malformation of body parts**
▢ **Nystagmus** (uncontrolled eye movements)
▢ **Arthrogryposis** (congenital stiffness of joints)

How long did the newborn stay in hospital? _______ days

First symptoms and diagnosis of Canavan Disease

The infant was completely normal before the onset of the first symptoms of the disease.

☐ **yes** ☐ **no**

What were the **symptoms at the onset** of the disease? (please check all that apply)

- ☐ **Developmental delay**
- ☐ **Low muscle tone** ('floppiness')
- ☐ **Feeding problems** (poor sucking)
- ☐ **Failure to thrive** (poor weight gain or growth)
- ☐ **Abnormal eye movements**
- ☐ **Poor vision**
- ☐ **Irritability**
- ☐ **Macrocephaly** (head too large)
- ☐ **Poor head control**
- ☐ **Sleep disturbances**
- ☐ **High muscle tone** ('stiffness')
- ☐ **Seizures** (epileptic fits)
- ☐ **Reduced movements of limbs**
- ☐ **Abnormal movements of limbs**
- ☐ **Poor hearing**
- ☐ **Change of behavior**
- ☐ **Microcephaly** (head too small)
- ☐ **Abnormal sweating**
- ☐ **Other** (please specify) ________________________________

First symptoms occurred at the age of _____ year(s) and _____ month(s)

The **diagnosis was confirmed** at the age of ____ year(s) and _____ month(s)

How was the diagnosis confirmed? (please check all that apply)

- ☐ **NAA (N-acetylaspartate) elevated in urine**
- ☐ **NAA elevated by MRS**
- ☐ **ASPA (aspartoacylase) activity decreased in skin cells**
- ☐ **ASPA activity decreased in blood cells**
- ☐ **Mutations in *ASPA gene***
- ☐ **Other** (please specify) ________________________________

In case mutations in *ASPA gene* were found, please specify the mutations:

____

____

Was **cerebrospinal fluid (CSF) protein** elevated? ☐ **yes** ☐ **no**

Are **biological samples** available (blood, cerebrospinal fluid, urine, tissue)?

☐ **yes** ☐ **no**

If biological samples are available, whom can we contact?

____

____

(Address of physician, hospital, medical center, or laboratory)

Psychomotor development

Eyesight and hearing

Was your child ever able to **follow an object visually**?

☐ **yes**, at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did you notice a **decline of visual abilities** in your child?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did you notice a **total loss of visual function** in your child?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child **able to hear**?

☐ **yes**, at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did you notice a **decline of hearing** in your child?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did you notice a **complete loss of hearing** in your child?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did you notice **a hypersensitivity to noise (startling)** in your child?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, was the **hypersensitivity to noise** lost?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Motor skills

Did your child gain **head control**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose head control**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **roll over from back to front or front to back**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to roll over?**

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **sit *without* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to sit *without* support?**

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **sit *with* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to sit *with* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child learn to **crawl** independently?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to crawl** independently?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child learn to **stand up** without support?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to stand up** independently?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child learn to **walk *with* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to walk *without* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child learn to **walk *without* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child lose the **ability to walk *with* support**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Fine motor skills

Did your child learn to **reach for an object**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child lose the **ability to reach for an object**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child **gain any voluntary hand function** (e.g. hold cup)?

☐ yes, at the age of ____ year(s) ____ month(s) ☐ no

If yes, did your child **lose hand function completely**?

☐ yes, at the age of ____ year(s) ____ month(s) ☐ no

Did your child learn to **transfer an object from hand-to-hand**?

☐ yes, at the age of ____ year(s) ____ month(s) ☐ no

If yes, did your child **lose the ability to transfer an object from hand-to-hand**?

☐ yes, at the age of ____ year(s) ____ month(s) ☐ no

Did your child l**earn to scrawl / draw**?

☐ yes, at the age of ____ year(s) ____ month(s) ☐ no

If yes, did your child **lose the ability to scrawl / draw**?

☐ yes, at the age of ____ year(s) ____ month(s) ☐ no

Development of language and other skills

Was your child able to **imitate noises**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to imitate noises**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child **able to communicate with you**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to communicate**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **speak single words** (i.e. mama, dada)?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, **how many words** was the child able to speak? ________ word(s)

If yes, did your child **lose the ability to speak words**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **speak single sentences**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to speak single sentences**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **count to five**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to count to five**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did the child **understand language**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to understand language**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **tell stories**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to tell stories?**

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child **able to read**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did the child **lose the ability to read?**

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child **able to write?**

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to write**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child able to **eat by himself / herself**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose the ability to eat by himself / herself**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was your child **toilet trained**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, did your child **lose toilet training skills**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Neurological findings

Was **spasticity** diagnosed (increased muscle tone or stiffness)?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did **seizures** (epileptic fits) occur?

☐ **yes, at first** at the age of ____ year(s) ____ month(s) ☐ **no**

Did **abnormal eye movements** (nystagmus) occur?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Were **irregularities of the optic nerve or retina** diagnosed?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, please specify: ____

Did involuntary (abnormal hyperactive) **movements of the body** occur?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Were **muscle reflexes (deep tendon reflexes) reduced** or have other **signs of impaired peripheral nerves** (i.e. reduced sensitivity to touch, pain or vibration) been diagnosed?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, please specify which: ____

Did you notice any **impairment of cognitive function** (i.e. poor concentration, forgetfulness, learning disability) in your child?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, please specify which: ____

Did your child suffer from **mood disorders** (i.e. depression, anxiety etc.)?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, please specify which: ______________________________

Other health problems

Did frequent **vomiting** occur?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child suffer from **constipation**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child suffer from **gastro-oesophageal reflux**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Was **surgery for prevention of gastric reflux** performed?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child need **gastric tube feeding**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child have **problems with excessive secretions / mucus**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did your child have **other problems with breathing**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

Did any **renal problems** occur?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, which renal problems occurred? ______________________________

Did your child have **other health problems** that have not been mentioned yet?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If other health problems occurred, please specify which:

____

____

Did your child receive **physical therapy or other supportive therapy**?

☐ **yes,** at the age of ____ year(s) ____ month(s) ☐ **no**

If yes, what **other types of training or support** did your child get? (please specify)

____

____

Which **type of school** (Kindergarten / regular school / special school) did your child visit?

Type of school: _______________ from the age of ______ to ______years

Type of school: _______________ from the age of ______ to ______years

How was the **evolution of the disease**? (please check all that apply)

▢ **stable**, from the age of _____ to _____ month(s) / (year(s)

▢ **intermittent**, from the age of _____ to _____ month(s) / (year(s)

▢ **slowly progressive**, from the age of _____ to _____ month(s) / (year(s)

▢ **quickly progressive**, from the age of _____ to _____ month(s) / (year(s)

▢ **none of the above**, from the age of _____ to _____ month(s) / (year(s)

Diagnostic workup

You may have reports of the test results. If you have them, please attach a copy of the results. Explanations of some procedures can be found in the attached information form.

Was an **(MRI) of the head** done? ▢ **yes** ▢ **no**

If yes, who can we contact for pictures or reports?

____

____

(Address of physician, hospital or medical center)

Were **nerve conduction studies** performed? ▢ **yes** ▢ **no**

If yes, was nerve conduction impaired? ▢ **yes** ▢ **no**

Was a hearing test performed (**auditory brainstem response**, BEARs)? ▢ **yes** ▢ **no**

If yes, the results were abnormal? ▢ **yes** ▢ **no**

Were **visual evoked potentials (VEPs)** tested? ▢ **yes** ▢ **no**

If yes, the results were abnormal? ▢ **yes** ▢ **no**

Was an **electroretinogram (ERG)** performed? ▢ **yes** ▢ **no**

If yes, the results were abnormal? ▢ **yes** ▢ **no**

Were **motor evoked potentials (MEPs)** tested? ▢ **yes** ▢**no**

If yes, the results were abnormal? ▢ **yes** ▢ **no**

Were **sensory evoked potentials (SEPs)** tested? ▢ **yes** ▢ **no**

If yes, the results were abnormal? ▢ **yes** ▢ **no**

Was an **electroencephalogram (EEG)** performed? ▢ **yes** ▢ **no**

If yes, the results were abnormal? ▢ **yes** ▢ **no**

Was **head circumference** measured? ☐ **yes** ☐ **no**

If head circumference was measured, which value was noted?

__________ inches / cm at the age of ______ year(s) and ______ month(s)

__________ inches / cm at the age of ______ year(s) and ______ month(s)

__________ inches / cm at the age of ______ year(s) and ______ month(s)

__________ inches / cm at the age of ______ year(s) and ______ month(s)

__________ inches / cm at the age of ______ year(s) and ______ month(s)

__________ inches / cm at the age of ______ year(s) and ______ month(s)

__________ inches / cm at the age of ______ year(s) and ______ month(s)

Development of seizures, language skills and visual abilities across age

Age	How often did seizures occur?	How were the language skills?	How were the visual abilities of your child?
1st year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
2nd year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
3rd year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
4th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
5th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
6th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**

Age	How often did seizures occur?	How were the language skills?	How were the visual abilities of your child?
7th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
8th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
9th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
10th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
11th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
12th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
13th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
14th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**
15th year	☐ **no seizures** ☐ **1-2 seizures / year** ☐ **less than 12 seizures / year** ☐ **more than 12 seizures / year**	☐ **normal for age** ☐ **minor difficulties** ☐ **major difficulties** ☐ **no verbal contact**	☐ **normal** ☐ **diminished, good spatial orientation** ☐ **poor, spatial orientation difficult** ☐ **no visual ability**

Age	How often did seizures occur?	How were the language skills?	How were the visual abilities of your child?
16th year	▢ **no seizures** ▢ **1-2 seizures / year** ▢ **less than 12 seizures / year** ▢ **more than 12 seizures / year**	▢ **normal for age** ▢ **minor difficulties** ▢ **major difficulties** ▢ **no verbal contact**	▢ **normal** ▢ **diminished, good spatial orientation** ▢ **poor, spatial orientation difficult** ▢ **no visual ability**
17th year	▢ **no seizures** ▢ **1-2 seizures / year** ▢ **less than 12 seizures / year** ▢ **more than 12 seizures / year**	▢ **normal for age** ▢ **minor difficulties** ▢ **major difficulties** ▢ **no verbal contact**	▢ **normal** ▢ **diminished, good spatial orientation** ▢ **poor, spatial orientation difficult** ▢ **no visual ability**
18th year	▢ **no seizures** ▢ **1-2 seizures / year** ▢ **less than 12 seizures / year** ▢ **more than 12 seizures / year**	▢ **normal for age** ▢ **minor difficulties** ▢ **major difficulties** ▢ **no verbal contact**	▢ **normal** ▢ **diminished, good spatial orientation** ▢ **poor, spatial orientation difficult** ▢ **no visual ability**

Experimental therapies

Did your child undergo **any experimental treatment**? ▢ **yes** ▢ **no**

If yes, which experimental treatment? (i.e. bone marrow transplantation, stem cell transplantation, medications or others)

Please specify which: ____

Which **positive effect**(s) of the experimental treatment did occur? (please specify)

____

____

Which **negative effect**(s) of the experimental treatment did occur? (please specify)

____

____

Did your child ever receive **any medication that was thought to ameliorate the course of the disease?**

(i.e. Glycerol triacetate (GTA), Lithium Citrate (Eskalith), Acetazolamide (Diamox) etc.)

▢ **yes** ▢ **no**

If yes, please specify which one(s): ____

Did your child ever take **any nutritional supplements that were thought to ameliorate the course of the disease**?

(i.e. Coenzyme Q10, Alpha-Lipoic Acid, Acetyl -L-Carnitine etc.)

▢ **yes** ▢ **no**

If yes, please specify which one(s): ____

Which of the medication had a **benefit to your** child?

____

____

Which **benefit** did it show? _______________________________

____

Which **negative side effect** did it have? _______________________

____

Which of the experimental medications showed **only negative side effects**?

____

Which side effects did it show?

____

____

Did your child take **medication for spasticity**?

▢ **yes** ▢ **no**

If yes, please specify which: _______________________________

If medication for spasticity was given, **when first** and **until when** was it given?

From ______ year(s) ______ month(s) **until** ______ year(s)_____ month(s)

Was **medication for seizures** given?

▢ **yes** ▢ **no**

If yes, please specify which: _______________________________

If medication for seizures was given, **when first** and **until when** was it given?

From ______ year(s) ______ month(s) **until** ______ year(s) _____ month(s)

Did your child t**ake medication for dystonia** (movement disorders)?

▢ **yes** ▢ **no**

If yes, please specify which: _______________________________

If medication for seizures was given, **when first** and **until when** was it given?

From ______ year(s) ______ month(s) **until** ______ year(s) _____ month(s)

What **other drugs** did your child take for prolonged periods of time?

(if possible please note also the duration of administration of this medication)

____

____

____

____

____

____

You may attach any comments that you consider to be important.

Thank you for your support!

You are helping other families.