

Trial Description

Title

Longitudinal Data Collection from Patients with Spinal Muscular Atrophy: The SMArtCARE Database

Trial Acronym

SMArtCARE

URL of the trial

<http://www.smartcare.de>

Brief Summary in Lay Language

The aim of SMArtCARE is to collect longitudinal “real-world data” on all available SMA patients independent of their actual treatment regime as disease-specific SMA registry. For this purpose, an online platform will be provided for SMA patients seen by health-care providers in German-speaking countries. Data are collected during routine patient visits and items for data collection are aligned with the international consensus for SMA registries. SMArtCARE is a joint initiative of neurologists, child neurologists, and patient organizations.

Brief Summary in Scientific Language

The aim of SMArtCARE is to collect longitudinal “real-world data” on all available SMA patients independent of their actual treatment regime as a disease-specific SMA registry. For this purpose, an online platform will be provided for SMA patients seen by health-care providers in German-speaking countries. Data are collected during routine patient visits and items for data collection are aligned with the international consensus for SMA registries. SMArtCARE collects data on motor function as well as changes in respiratory function, nutritional status and orthopedic symptoms. To evaluate changes in motor function, standardized physiotherapeutic assessments are performed. SMArtCARE is a joint initiative of neurologists, child neurologists, and patient organizations.

Do you plan to share individual participant data with other researchers?

[---]*

Description IPD sharing plan

[---]*

Organizational Data

- DRKS-ID: **DRKS00012699**
- Date of Registration in DRKS: **2018/08/09**
- Date of Registration in Partner Registry or other Primary Registry: [---]*
- Investigator Sponsored/Initiated Trial (IST/IIT): **yes**
- Ethics Approval/Approval of the Ethics Committee: **Approved**
- (leading) Ethics Committee Nr.: **56/18 , Ethik-Kommission der Albert-Ludwigs-Universität Freiburg**

Secondary IDs

Health condition or Problem studied

- Free text: **5q spinal muscular atrophy (SMA)**
- ICD10: **G12.0 - Infantile spinal muscular atrophy, type I [Werdnig-Hoffman]**

Interventions/Observational Groups

- Arm 1: **The aim of SMARTCARE is to collect longitudinal “real-world data” on all available SMA patients independent of their actual treatment regime as a disease-specific SMA registry. For this purpose, an online platform will be provided for SMA patients seen by health-care providers in German-speaking countries. Data are collected during routine patient visits and items for data collection are aligned with the international consensus for SMA registries. SMARTCARE collects data on motor function as well as changes in respiratory function, nutritional status and orthopedic symptoms. To evaluate changes in motor function, standardized physiotherapeutic assessments are performed.**

Characteristics

- Study Type: **Non-interventional**
- Study Type Non-Interventional: **Observational study**
- Allocation: **Single arm study**
- Blinding: [---]*
- Who is blinded: [---]*
- Control: **Uncontrolled/Single arm**
- Purpose: **Other**
- Assignment: **Single (group)**
- Phase: **N/A**
- Off-label use (Zulassungsüberschreitende Anwendung eines Arzneimittels): [---]*

Primary Outcome

The aim of SMArtCARE is to collect data from all SMA patients during routine patient visits. SMArtCARE collects data on motor function as well as changes in respiratory function, nutritional status and orthopedic symptoms. To evaluate changes in motor function, standardized physiotherapeutic assessments are performed.

Secondary Outcome

na

Countries of recruitment

- **DE Germany**
- **AT Austria**
- **CH Switzerland**

Locations of Recruitment

- University Medical Center **Klinik für Neuropädiatrie und Muskelerkrankungen, Freiburg im Breisgau**
- Medical Center **DRK Kliniken Berlin Westend - Klinik für Kinder- und Jugendmedizin, Berlin**
- University Medical Center **Universitätsklinikum Essen, Klinik für Kinderheilkunde - Neuropädiatrie, Essen**
- University Medical Center **Universitätsklinikum Essen, Neurologische Klinik und Poliklinik, Essen**
- Medical Center **Asklepios Klinik Nord Hamburg - Neuropädiatrie, Hamburg**
- University Medical Center **Medizinische Hochschule Hannover, Klinik für Neurologie, Hannover**
- Medical Center **Klinikum Kassel, Neuropädiatrie, Kassel**
- University Medical Center **Universitätsmedizin Leipzig, Klinik und Poliklinik für Neurologie, Leipzig**
- University Medical Center **Universitätsmedizin Mannheim, Neurologische Klinik, Mannheim**
- Medical Center **Klinikum der Universität München, Friedrich-Baur-Institut, München**
- University Medical Center **Universitätsklinikum Tübingen, Kinderklinik Abteilung III, Tübingen**
- University Medical Center **Universitätsklinikum Würzburg, Kinderklinik und Poliklinik, Sozialpädiatrisches Zentrum, Neuropädiatrie, Würzburg**
- University Medical Center **Universitätsklinikum Würzburg, Neurologische Klinik und Poliklinik, Würzburg**

- Medical Center **Med. Campus Universität Oldenburg, Klinik für neurologische Intensivmedizin und Frührehabilitation, Oldenburg**
- University Medical Center **Charité - Universitätsmedizin Berlin Campus Virchow Klinikum, Berlin**
- Medical Center **Klinik für Kinder- und Jugendmedizin Ruhruniversität Bochum im St. Josef Hospital, Bochum**
- University Medical Center **Universitätsklinikum Carl Gustav Carus Dresden an der Technischen Universität Dresden - Neuropädiatrie, Dresden**
- University Medical Center **Universitätsklinikum Carl Gustav Carus Dresden an der Technischen Universität Dresden - Neurologie, Dresden**
- University Medical Center **Universitätsklinikum Gießen und Marburg GmbH Klinikum der Justus-Liebig-Universität - Neuropädiatrie, Gießen**
- University Medical Center **Universitätsmedizin Göttingen - Neurologie, Göttingen**
- University Medical Center **Universitätsklinikum Halle - Neurologie, Halle Saale**
- University Medical Center **Universitätsklinikum Hamburg-Eppendorf Zentrum für Geburtshilfe, Kinder- und Jugendmedizin, Hamburg**
- University Medical Center **Universitätsklinikum des Saarlandes - Klinik für Allgemeine Pädiatrie und Neonatologie, Homburg**
- University Medical Center **Universitätsklinik für Pädiatrie I Department für Kinder - und Jugendheilkunde, Innsbruck**
- Medical Center **Klinik für Neuropädiatrie Sozialpädiatrisches Zentrum, Jena**
- Medical Center **Hans-Berger-Klinik für Neurologie, Jena**
- Medical Center **Kliniken der Stadt Köln gGmbH Kinderkrankenhaus, Köln**
- Medical Center **Ordensklinikum Linz GmbH Barmherzige Schwestern - Neuropädiatrie, Linz**
- University Medical Center **Kepler Universitätsklinikum Linz - Neurologie, Linz**
- Medical Center **Technische Universität München Klinikum rechts der Isar - Neurologie, München**
- University Medical Center **Universitätsklinikum Münster - Neuropädiatrie, Münster**
- Medical Center **Landesklinikum Baden-Möding - Neuropädiatrie, Möding**
- Medical Center **Klinikum Stuttgart Olgaspedial - Neurologie, Stuttgart**
- Medical Center **Kantonsspital St. Gallen, St. Gallen**
- Medical Center **Klinikum Wels-Grieskirchen GmbH - Neuropädiatrie, Wels**
- Medical Center **DKD Helios Klinik Wiesbaden - Neurologie, Wiesbaden**
- Medical Center **Klinikum Klagenfurt am Wörthersee - Neuropädiatrie, Klagenfurt**
- Medical Center **Universitätsklinikum Augsburg - Mutter-Kind-Zentrum Schwaben, Augsburg**
- Medical Center **Landeskrankenhaus Bregenz - Neuropädiatrie, Bregenz**
- Medical Center **Kaiser-Franz-Josef Spital mit G.v. Preyer'sches Kinderspital, Wien**

- Medical Center **Medizinische Hochschule Hannover - Neuropädiatrie, Hannover**
- University Medical Center **Universitätsmedizin Göttingen - Neuropädiatrie, Göttingen**
- University Medical Center **Universitätsklinikum Heidelberg, Heidelberg**
- University Medical Center **Universitätsklinikum Schleswig-Holstein - Neurologie, Kiel**
- University Medical Center **Universitätsklinikum Bonn - Neuropädiatrie, Bonn**
- University Medical Center **Universitätsklinikum Erlangen - Neuropädiatrie, Erlangen**
- University Medical Center **Universitätsklinikum Erlangen - Neurologie, Erlangen**
- Medical Center **Klinikum Klagenfurt am Wörthersee - Neurologie, Klagenfurt**

Recruitment

- Planned/Actual: **Actual**
- (Anticipated or Actual) Date of First Enrollment: **2018/07/15**
- Target Sample Size: **1000**
- Monocenter/Multicenter trial: **Multicenter trial**
- National/International: **International**

Inclusion Criteria

- Gender: **Both, male and female**
- Minimum Age: **no minimum age**
- Maximum Age: **no maximum age**

Additional Inclusion Criteria

- **All patients with genetically confirmed 5q SMA with residence in German-speaking regions.**
- **Informed consent provided by patient/caregiver**

Exclusion criteria

- **Further types of SMA (non 5q SMA)**
- **Participation in an AMG trial**

Addresses

- **Primary Sponsor**
Universitätsklinikum Bonn Eltern-Kind-Zentrum (ELKI)
Mr. Prof. Dr. Janbernd Kirschner

Primary Sponsor

Universitätsklinikum Bonn Eltern-Kind-Zentrum (ELKI)

Mr. Prof. Dr. Janbernd Kirschner

53127 Bonn

Germany

Telephone: [---]*

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URL: [---]*

■ **Contact for Scientific Queries**

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■ **Contact for Public Queries**

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URL: **www.uniklinik-freiburg.de**

Sources of Monetary or Material Support

■ **Commercial (pharmaceutical industry, medical engineering industry, etc.)**

Biogen International GmbH

6300 Zug

Switzerland

Telephone: [---]*

Fax: [---]*

Commercial (pharmaceutical industry, medical engineering industry, etc.)

Biogen International GmbH

6300 Zug

Switzerland

Telephone: [---]*

Fax: [---]*

E-mail: [---]*

URL: [---]*

Status

- Recruitment Status: **Recruiting ongoing**
- Study Closing (LPLV): [---]*

Trial Publications, Results and other documents

- Paper **SMartCARE - A platform to collect real-life outcome data of patients with spinal muscular atrophy** (Pechmann A et al., Orphanet Journal of Rare Diseases, 2019)
- Paper **Treatment with Nusinersen - Challenges Regarding the Indication for Children with SMA Type 1** (Pechmann A et al., Journal of neuromuscular diseases, 2020)
- Paper **Zur Gentherapie der Spinalen Muskelatrophie mit Onasemnogene Abeparvovec. Stellungnahme der Gesellschaft für Neuropädiatrie** (Kirschner J et al., Monatsschrift Kinderheilkunde, 2020)
- Paper **Evaluation of Children with SMA Type 1 Under Treatment with Nusinersen within the Expanded Access Program in Germany** (Pechmann A et al., Journal of Neuromuscular Diseases, 2018)

Please note:

There are additional attributes available concerning this trial. To open an extended view please [click here](#).