Data items SMArtCARE

	Data items	Answers	If yes:		Comments

Enrolment

Enrolment	Date enrolled				automatically generated
	Date of consent				
	Genetically proven 5q SMA				
	First name				
	Last name				
	Last name at birth				
	Date of birth				
	Country of birth				
	City of birth				
	Country of residence	Austria Germany Switzerland Other (specify)			
	Patient identification number				internal hospital patient ID
	Email address				
	Gender	Male Female Undifferentiated Unknown			

Baseline

Genetic Test Result	Do you have a copy of the genetic report?	Yes No	Date of genetic report		
			Name of genetic institute		
			Type of mutation	Homozygous deletion involving exon 7 of SMN1 Heterozygous deletion and heterozygous point mutation (compound heterozygotes) Other (specify)	
	SMN2 copy number performed?	Yes No	*SMN2* copy number	1, 2, 3, 4, 5, 6, 7, 8 other (specify)	
			Molecular testing approach of *SMN2*	MLPA Sequence analysis Other (specify)	
	Was diagnosis made pre-symptomatically?	Yes No	Screening type	Newborn screening Positive family history Other (specify)	
	Any other family member affected?	Yes No Unknown			Kinship as auto-complete
Clinical diagnosis	First symptoms or signs leading to suspect SMA. Please specify.				
	Age at symptom onset	Prenatal At birth Within the first 4 weeks At the age of (specify)			
	Sitting without support Crawl on hands and knees Standing without support Walking without	Never able Gained Gained and lost	Age gained Age lost		Crawl on hands on knees only in patients < 18 years of age

	support Climb stairs				
	Wheelchair use?	No Part-time Full-time	Start date of wheelchair use		Wheelchair use only in patients > 2 years of age
	Does the patient receive ventilator support?	Yes No	Type of ventilator support	Non-invasive Invasive	
				Start date of ventilator support	
	Does the patient use a gastric or nasal feeding tube?	Yes – supplementary e.g. for fluids Yes - exclusively fed by tube No	Start date of tube feeding		
	Scoliosis surgery?	Yes No	Date of surgery		
			Surgery technique	Arthrodesis Growing rods Other (specify)	
	Any other pre-existing illness?	Yes No Unknown	Add pre-existing illness		ICD 10 code
Registries, clinical trials	Is the patient currently or was previously included in a clinical trial?	Yes No	Currently included Previously included		
			Name of trial		
			Name of drug		
			Start date		
			End date		
	Is the patient currently enrolled in other registries?	Yes No Unknown	Specify		

Medical Assessment

	Visit date				
	Age at visit				automatically filled, years and months
	Physician's first name				
	Physician's last name				
Pulmonary	Does the patient receive ventilator support?	Yes No	Type of ventilation	Non-invasive Invasive	
				Date of change	
			Frequency of use	Daily use Occasional use Use when ill only	
			Time of ventilator use	Night (during sleep) Intermittent day time and continuous at night Continuous (>16h/day) Intermittent with acute illnesses	
			Hours per day	1-24	
			Start of ventilator use (date) Ongoing End of ventilator use (date)		
	Assistance in airway clearance and secretion mobilisation	Yes No	Type of airway clearance and secretion mobilisation	Suction Chest physiotherapy Cough assist device Other (specify)	
			Frequency of use	Daily use Occasional use Use when ill only	
	Pulmonary function performed?	Yes No	Date of pulmonary function		
			FVC percentage		

			FVC litre		
			Peak cough flow		
	Vaccination performed?	Yes No	RSV	Date of last vaccination	
			Influenza	Date of vaccination	
			Pneumococcus	Date of vaccination	
Nutrition	Does the patient use a gastric or nasal feeding tube?	No Yes - exclusively fed by tube Yes – supplementary e.g. for fluids	Start of tube feeding (date) Ongoing End of tube feeding (date)		
	Swallowing?	Normal With difficulties			
	Chewing?	Normal With difficulties			Only in patients > 1 year of age
Orthopaedics	Does the patient have scoliosis?	Yes No	Was new x-ray performed?	Yes No	
			Date of x-ray		
			Cobb angle		
	Orthopaedic surgery since last visit?	Yes No	Type of orthopaedic surgery	Scoliosis surgery Other types of orthopaedic surgery (specify)	
			Surgery technique	Arthrodesis Growing rods Other (specify)	
	Does the patient suffer pain regularly?	Yes No Unknown	Pain intensity	Mild Moderate Severe	
			Localisation		
	Does the patient experience fatigue?	Yes No Unknown	Specify		
Hospitalisation	Planned hospitalisation since last visit (except for	Yes No Unknown	Admission date Ongoing Discharge date		

	treatment administration)?				
			Reason for hospitalisation	Sleep study Placement of gastric tube Orthopaedic surgery Other (specify)	
Therapy	Therapy interventions?	Physiotherapy Feeding / Speech Occupational Therapy Other (specify)			
	Orthoses?	Yes No	Type of orthoses	Ankle-foot Orthoses (AFOs) Supramalleolar Orthoses (SMO) Orthotics/shoe inserts Knee-ankle-foot Orthoses (KAFOs) Hand splints Other (specify)	
			Orthotic Type of Use	Functional, Day time only Resting Day time Resting Night time	
			Frequency (times per week)	1-7	
	Devices?	Yes No	Type of devices	Standing Frame Positioning Device (Seating system) Therapy bicycle Galileo Other (specify)	
			Frequency (times per week)	1-7	
	Wheelchair use?	No Part-time Full-time	Type of wheelchair	Manual wheelchair Power wheelchair	Only in patients > 2 years of age
			Start date		
Medication	Is the patient on any	Yes	Name of drug		

	approved medication for SMA	No			
			Start date Ongoing End date		
	Other medication taken on a regular basis	Yes No	Name of medication		
			Start date Ongoing End date		
Clinical Trial	Is the patient currently included in a clinical trial?	Yes No Unknown	Name of trial		
			Name of drug		
			Start date		
Best current motor function	Any changes in motor milestones?	No Improvement Worsening	Best current motor function	Sitting without support Crawl on hands and knees Standing without support Walking without support Climb stairs None of the above	Crawl on hands and knees only in patients < 18 years of age
			Age gained of new motor milestone		
			Age loss of previous motor milestone		
Upper limb function	Achieve useful function of hands (e.g. hold a pen or pick pennies up from a table)	Yes No			
	Raise hands to mouth (in a sitting position)	Yes No			
	Reach overhead (in a sitting position)	Yes No			

HINE					In patients < 12 years of age
Clinical examination	Height (cm)				Percentile auto-calculated in patients < 18 years of age
	Body weight (kg)				Percentile auto-calculated in patients < 18 years of age
	Head circumfereance (cm)				Percentile auto-calculated in patients < 3 years of age
	BMI				Auto-calculated
	Skin	Normal Abnormal Not examined	Specify		
	HEENT	Normal Abnormal Not examined	Specify		
	Pulmo	Normal Abnormal Not examined	Specify		
	Heart	Normal Abnormal Not examined	Specify		
	Abdomen	Normal Abnormal Not examined	Specify		
	Neurology	Symptoms related to SMA Neurological findings not related to SMA Not examined	Specify		
	Are any contractures present?	Yes No	Type of limitation	Minimal (no limitations to function) Moderate (some limitations, adaptions necessary) Severe (imposing limits to function)	
			Description of contractures / localisation		

Laboratory	Laboratory testing performed?	Yes No	Any abnormalities of clinical significance?	Yes (specify) No	
Neuro-physiology (optional)	Motor nerve conduction velocity (m/s)	Ulnar Median Tibialis			
	F-wave latency (ms)	Ulnar Median Tibialis			
	CMAP amplitude (mV)	Ulnar Median Tibialis			
PROM	General impression	Much improved Minimally improved No changes Minimally worse Much worse	Specify		In patients > 12 years of age: description of the impression in comparison to 12 months ago. In patients <=12 years of age: description of the impression in comparison to 6 months ago. In patients < 12 months of age: description of the impression in comparison to 3 months ago.
	Motor function	Much improved Minimally improved No changes Minimally worse Much worse	Specify		
	Respiratory function	Much improved Minimally improved No changes Minimally worse Much worse	Specify		

Adverse Events

Adverse events	Date recorded				automatically generated
	Has there been any adverse event since the last visit?	Yes No			
	Has there been unplanned or prolonged hospitalisation?	Yes No	Type of unexpected event	Respiratory tract infection Hydrocephalus Epileptic seizure Post lumbar puncture syndrome Other (specify)	
			Medra code of acute event		
			Description of acute event		
			Admission date Ongoing Discharge date		
			Drug treatment for post lumbar puncture syndrome necessary?	Yes No Unknown	
			Type of drug treatment for post lumbar puncture syndrome	Analgesics Caffeine Other (specify)	
			Is the adverse event related to drug treatment?	No Possibly Yes	If “possibly” or “yes”: Generation of a standardized form to report drug related adverse events to the respective authority
			Name of drug		
	Any unexpected events without hospitalisation?	Yes No	Type of unexpected event	Respiratory tract infection Hydrocephalus Epileptic seizure Post lumbar puncture syndrome Other (specify)	

			Medra code of acute event		
			Description of acute event		
			Start date Ongoing End date		
			Post lumbar puncture syndrome?	Drug treatment for post lumbar puncture syndrome necessary?	
			Type of drug treatment for post lumbar puncture syndrome	Analgesics Caffeine Other (specify)	
			Is the adverse event related to drug treatment?	No Possibly Yes	If “possibly” or “yes”: Generation of a standardized form to report drug related adverse events to the respective authority
			Name of drug		

Nusinersen

Nusinersen	Date of treatment				
	Care Setting	Outpatient Inpatient (overnight)			
	Ventilator support during LP	Yes No	Type of ventilator support	General anaesthesia Patient's ventilator device	
	Anaesthesia or sedation	Yes No	Drug		
			Application	Intranasal Intravenous Peroral Other (specify)	
	Use of	Ultrasound Fluoroscopy/radioscopy CT-Scan	Radiation dose of CT scan		

		Other (specify)			
	Duration of procedure				
	Adverse events during procedure	Yes No	Specify		

End of data collection

End of data collection	Date recorded				automatically generated
	Has the patient withdrawn the consent?	Yes No			
	Is the patient deceased?	Yes No	Date of death		
			Cause of death		ICD 10 code
	Other reason for end of data collection				