

Recommendations for the evaluation of patients with SMA

	Patient visits (days)					Ongoing every 6 months (under SMA-specific treatment every 4 months)
	1	14	28	63	180	
Baseline data	X					
Current medical history and clinical examination* including motor milestones	X	X	X	X	X	X
Case Report Form Nusinersen	X	X	X	X	X	X
Case Report Forms Zolgensma	X				X	X
Documentation of Risdiplam	X			X	X	X
Physiotherapeutic assessments***: CHOP INTEND <ul style="list-style-type: none"> All children < 2 years of age All patients > 2 years of age without ability to sit HFMSE <ul style="list-style-type: none"> All patients > 2 years of age with ability to sit If CHOP INTEND score >50: CHOP INTEND and HFMSE**** If CHOP INTEND score >60: HFMSE instead of CHOP INTEND RULM <ul style="list-style-type: none"> All patients > 2 years of age with ability to sit in a wheelchair 6-MWT <ul style="list-style-type: none"> All ambulant patients > 3 years of age 	X			X**	X	X
ALS Functional Rating Scale in adult patients	X				X	X
Pulmonary function*****	X				X	X
Documentation of adverse events	Ongoing					

black = untreated patients; grey = patients under SMA-specific therapy

* Conducted by neuropaediatrician or neurologist

** Only for therapy with Zolgensma

*** To be conducted by appropriately trained physiotherapists. Definition of sitting and walking ability according to WHO criteria

**** In patients < 2 years of age, HFMSE is not yet possible due to cooperation. In patients < 2 years of age with CHOP INTEND score > 60, the motor part of the Bayley-III Scales should therefore be performed in addition to CHOP INTEND.

***** If the patient is sufficiently cooperative