

Recommendations for the evaluation of paediatric 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					
Medical assessment¹ including motor milestones	X	X	X	X	X	X
Case Report Form Nusinersen	X	X	X	X	X	X
Case Report Forms Zolgensma²	X				X	
Documentation of Risdiplam³	X			X	X	X
Physiotherapeutic assessments⁴: CHOP INTEND <ul style="list-style-type: none"> All children < 2 years of age All patients > 2 years of age without ability to sit BAYLEY III Scale (≤ 2 years) <ul style="list-style-type: none"> Motor function scale: if CHOP INTEND score >60 HFMSE (> 2 years) <ul style="list-style-type: none"> All patients with ability to sit If CHOP INTEND score >50: CHOP INTEND and HFMSE If CHOP INTEND score >60: HFMSE instead of CHOP INTEND RULM (> 2 years) <ul style="list-style-type: none"> All patients with ability to sit in a wheelchair 6-MWT (> 2 years) <ul style="list-style-type: none"> All patients with ability to walk 	X		X ⁵	X	X	X
Bayley III Scale (Cognition, motor function and language)⁶						Once at the age of 24 months
ALS Functional Rating Scale in adult patients	X				X	X
Pulmonary function⁷	X				X	X
Documentation of adverse events	Ongoing					

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

1 To be conducted by neuropaediatricians

2 On day 1, the case report form "Zolgensma Day of treatment" must be completed. On day 180, the case report form "Zolgensma Follow-Up" must be completed

3 The documentation of the Risdiplam therapy is part of the Medical Assessment

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

5 Only for therapy with Zolgensma

6 For children who are able to sit in a wheelchair and raise their arms independently against gravity

7 If the patient is sufficiently cooperative