NYE METODER

Anmodning om vurdering av legemiddel i Nye metoder

Skjema for leverandører

En leverandør som ønsker offentlig finansiering av et legemiddel/legemiddelindikasjon i den norske spesialisthelsetjenesten, skal anmode om vurdering i Nye metoder ved å fylle ut dette skjemaet.

Utfylt anmodningsskjema sendes til Nye metoder: nyemetoder@helse-sorost.no

Leverandøren skal på anmodningstidspunktet både ha et forslag til type helseøkonomisk analyse og en plan for når de leverer dokumentasjonen. Merk at dokumentasjon i henhold til oppdraget fra Bestillerforum for nye metoder må leveres inn senest 12 måneder etter anmodningstidspunktet.

Hele anmodningsskjemaet skal fylles ut. Mer informasjon og veiledning finnes i artikkelen For leverandører (nyemetoder.no)

Merk: Skjemaet vil bli publisert i sin helhet på nyemetoder.no.

Innsender er klar over at skjemaet vil bli publisert i sin helhet (må krysses av): ☒

Fyll ut dato for innsending av skjema: 29.052026

1 Kontaktopplysninger	
1.1 Leverandør (innehaver/søker av markedsføringstillatelse i Norge)	Novartis Norge AS
1.2 Navn kontaktperson	Karen Marie Hanevik-Heggberget
1.3 Stilling kontaktperson	Value & Access Manager
1.4 Telefon	98445564
1.5 E-post	karen_marie.hanevik-heggberget@novartis.com
Ekstern representasjon - vedlegg fullmakt	
1.6 Navn/virksomhet	Klikk eller trykk her for å skrive inn tekst.
1.7 Telefon og e-post	Klikk eller trykk her for å skrive inn tekst.

2 Legemiddelinformasjon og indikasjon	
2.1 Hva gjelder anmodningen? *Kryss av for hva anmodningen gjelder*	Et nytt virkestoff ☐ En indikasjonsutvidelse / ny indikasjon ☐ En ny styrke eller formulering ☒
2.2 Hvilken indikasjon gjelder anmodningen?	Itvisma er indisert til behandling av 5q spinal muskelatrofi (SMA) med en bi-allelisk mutasjon i *SMN1*-genet, hos pasienter fra 2 år og oppover.

Indikasjonen skal oppgis på norsk. Hvis prosess for godkjenning pågår, oppgi også indikasjon på engelsk. *Merk: Leverandør skal anmode om vurdering av hele indikasjonen som de har fått godkjent eller søker om godkjenning for. Dersom leverandør foreslår en avgrensning til undergrupper, må dette begrunnes og leverandør må levere dokumentasjonen som trengs for å foreta en vurdering av undergruppen i tillegg til dokumentasjonen for hele indikasjonen.*	Nedenfor er engelsk indikasjonsordlyd: Itvisma is indicated for the treatment of 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene in patients 2 years of age and older
2.3 Handelsnavn	Itvisma®
2.4 Generisk navn/virkestoff	Onasemnogene abeparvovec (intrathecal (OAV101, IT))
2.5 ATC-kode	M09AX09
2.6 Administrasjonsform og styrke *Oppgi også forventet dosering og behandlingslengde* *Skriv kort*	1.2 × 10^14 vector genomes solution for injection The drug is administered as an intrathecal injection and is a one-time, fixed-dose gene therapy, with no upper weight limit, designed to treat SMA in patients aged 6 months and older, eliminating the need for lifelong treatment or dose adjustments. Patients will receive of 1.2 × 10^14 vg of onasemnogene abeparvovec IT
2.7 Farmakoterapeutisk gruppe og virkningsmekanisme. *Skriv kort*	Farmakoterapeutisk gruppe: Other drugs for disorders of the musculo-skeletal system Itvisma introduces a functional copy of the SMN1 gene into motor neurons using a non-replicating recombinant AAV9 capsid, providing sustained SMN protein expression to address the genetic root cause of SMA.

3 Historikk – virkestoff og indikasjon

3.1 Har Nye metoder behandlet metoder med det aktuelle virkestoffet tidligere? *Hvis ja, oppgi ID-nummer til metoden/metodene i Nye metoder*	Ja ☒ Nei ☐ ID-nummer: ID2019_006
3.2 Er du kjent med om andre legemidler/virkestoff er vurdert i Nye metoder til samme indikasjon?	Ja ☒ Nei ☐

Hvis ja, oppgi ID-nummer til metoden/metodene i Nye metoder	ID-nummer: Nusinersen: ID2017_001, ID2020_031 Risdiplam: ID2020_104, ID2021_088, ID2024_042, ID2025_010
3.3 Er du kjent med om det er gjennomført en metodevurdering i et annet land som kan være relevant i norsk sammenheng? *Hvis ja, oppgi referanse*	Ja ☐ Nei ☒ Referanse: Part of HTAR-JCA

4 Status for markedsføringstillatelse (MT) og markedsføring

4.1 Har legemiddelet MT i Norge for en eller flere indikasjoner? *Hvis ja - skriv inn dato for norsk MT for den første indikasjonen*	Ja ☐ Nei ☒ Dato for MT for første indikasjon: Klikk eller trykk for å skrive inn en dato.
4.2 Markedsføres legemiddelet i Norge?	Ja ☐ Nei ☒
4.3 Har legemiddelet MT i Norge for anmodet indikasjon? *For alle metoder: Fyll ut prosedyrenummer i EMA (det europeiske legemiddelbyrået)*	MT i Norge: Ja ☐ Nei ☒ Prosedyrenummer i EMA: EMEA/H/C/0006498 (EMA product number)
Hvis metoden ikke har MT i Norge, fyll ut forventet tidspunkt (måned/år) for CHMP opinion i EMA. *Hvis metoden har MT i Norge, fyll ut dato for MT*	**Hvis metoden ikke har MT**: Forventet tidspunkt for CHMP opinion i EMA (måned/år): 23.04.2026 Forventet tidspunkt for markedsføringstillatelse (MT) for den aktuelle indikasjonen i Norge (måned/år): 06.08.2026
	Hvis metoden har MT: Dato for MT i Norge for den aktuelle indikasjonen: Klikk eller trykk for å skrive inn en dato.
4.4 Har legemiddelet en betinget markedsføringstillatelse for anmodet indikasjon?	Ja ☐ Nei ☒ Beskrivelse:

Hvis ja, fyll ut en beskrivelse av hva som skal leveres til EMA og når.	
4.5 Har anmodet indikasjon vært i «accelerated assessment» hos EMA?	Ja ☐ Nei ☒
4.6 Har legemiddelet «orphan drug designation» i EMA? *Hvis ja, fyll ut dato*	Ja ☒ Nei ☐ Dato for «orphan drug designation»: 19.06.2015

5 Ordning for forenklet vurdering av PD-(L)1-legemidler

5.1 Er legemiddelet registrert i Nye metoders ordning «Forenklet vurdering av PD-(L)1-legemidler"?	Ja ☐ Nei ☒

6 Sammenlignbarhet og anbud

6.1 Finnes det andre legemidler med lignende virkningsmekanisme og /eller tilsvarende effekt til den aktuelle indikasjonen?	Ja ☒ Nei ☐ Kommentar: Nusinersen (Spinraza) Risdiplam (Evrysdi) Onasemnogene abeparvovec IV (Zolgensma) Dette er legemidler med lignende effekt, men ulike virkningsmekanismer og/eller administrasjonsformer. Zolgensma har ulik indikasjon (populasjon (alder))
6.2 Vurderer leverandør at legemiddelet i anmodningen er sammenlignbart med et eller flere andre legemidler som Nye metoder har besluttet å innføre til den samme indikasjonen? *Hvis ja, hvilke(t)? Oppgi ID-nummer på metoden/metodene i Nye metoder*	Ja ☒ Nei ☐ Legemiddel og ID-nummer: Nusinersen: ID2017_001, ID2020_031 Risdiplam: ID2021_088, ID2024_042, ID2025_010 Onasemnogene abeparvovec IV : ID2019_006 (not overlapping population (age))
6.3 Er det eksisterende anbud på terapiområdet som kan være aktuelt for legemiddelet?	Ja ☐ Nei ☒ Kommentar: Klikk eller trykk her for å skrive inn tekst.

7 Nordisk samarbeid JNHB (Joint Nordic HTA-bodies)	
7.1 Er anmodet indikasjon aktuell for utredning i det nordiske HTA-samarbeidet JNHB? *Hvis nei, begrunn kort*	Ja ☐ Nei ☒ Begrunnelse: We have chosen not to apply for JNHB because it doesn't fullfill criteria for JNHB: there is different clinical practice in the Nordic countries and deviating economic models between the countries.

8 Europeisk samarbeid om vurdering av relativ effekt og sikkerhet (HTAR)	
8.1 Er anmodet legemiddel/indikasjon omfattet av regelverket for utredning av relativ effekt og sikkerhet i europeisk prosess (HTAR)? *Hvis ja, fyll ut dato for søknad om MT til EMA*	Ja ☒ Nei ☐ Dato for søknad til EMA: 17.12.2025

9 Helseøkonomisk dokumentasjon og forslag til helseøkonomisk analyse	
9.1 Hvilken type helseøkonomisk analyse foreslår leverandøren? *F.eks. kostnad-per-QALY analyse eller kostnadsminimeringsanalyse.* *Begrunn forslaget*	Kostnad-per-QALY analyse Reasoning: SMA is a lifelong condition and Itvisma is intended to provide long-term and potentially lifelong benefits. With a cost-utility assessment, explicit valuation of long-term health effects is considered. Itvisma is a one-time gene therapy with a different mechanism of action compared to current treatments and a durable effect is expected. There is evidence that the effect of other chronic SMA treatments **in some patients** may wane over time. A cost-utility analysis allows DMP to assess value of a gene therapy transparently under uncertainty, by explicitly modelling assumptions, testing alternative scenarios, and reflecting differences in accumulated QALYs over a lifetime horizon. On the other hand, a cost-minimisation analysis is based on the assumption that there is equivalence between treatments across the entire disease course. Due to lack of long-term data, this assumption might not be true as the patient undergoes further chronic treatment. It will not be able to capture relevant differences in treatment burden, stability of motor function, caregiver burden, and broader utility effects

	associated with a one-time intervention. For instance, with Spinraza, the burden of treatment with several intrathecal injections each year over a lifetime as described in ID2024_042 is unfavorable in comparison to a one-time treatment such as Itvisma. Even if short-term clinical outcomes appear similar, this does not imply equal lifetime utility or equal value. For these reasons, a cost-utility analysis is the appropriate and methodologically robust framework for assessing Itvisma in line with DMP's established principles for severe, lifelong conditions.
9.2 Pasientpopulasjonen som den helseøkonomiske analysen baseres på, herunder eventuelle undergrupper.	Treatment of 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the survival motor neuron 1 (SMN1) gene in patients 2 years of age and older
9.3 Hvilken dokumentasjon skal ligge til grunn? (H2H studie, ITC, konstruert komparatorarm etc.) *Angi det som er relevant med tanke på hvilken type analyse som foreslås.*	Relevant clinical documentation is discussed and evaluated in the JCA report.
9.4 Forventet legemiddelbudsjett i det året med størst budsjettvirkning i de første fem år.	SMA is a rare disease and most patients eligable for treatment will be patients already treated with Evrysdi or Spinraza. Because of this budget impact is most likely small or none at all.
9.5 Forventet tidspunkt (måned og år) for levering av dokumentasjon til Direktoratet for medisinske produkter og/eller Sykehusinnkjøp HF. *Tidspunkt må oppgis*	09.2026

10 Sykdommen og eksisterende behandling	
10.1 Sykdomsbeskrivelse for aktuell indikasjon *Kort beskrivelse av sykdommens patofysiologi og klinisk presentasjon / symptombilde, eventuelt inkl. referanser*	Spinal muscular atrophy (SMA) is a rare, genetic neuromuscular disorder characterized by progressive loss of motor neurons, leading to muscle weakness, paralysis, and, in its more severe forms, premature death if untreated. The most common form, 5q SMA, results from biallelic mutations or deletions in the survival motor neuron 1 (SMN1) gene on chromosome 5q, causing deficiency of survival motor neuron (SMN) protein essential for motor neuron function, maintenance, and survival.
10.2 Fagområde *Angi hvilket fagområde som best beskriver metoden*	Velg fagområde fra menyen: Nevrologi
10.3 Kreftområde *Hvis metoden gjelder fagområdet Kreftsykdommer, angi hvilket kreftområde som er aktuelt*	Velg kreftområde fra menyen: Velg et element.
10.4 Dagens behandling *Nåværende standardbehandling i Norge, inkl. referanse*	https://www.nyemetoder.no/metoder/risdiplam-evrysdi-indikasjon-ii/, https://www.nyemetoder.no/metoder/risdiplam-evrysdi-indikasjon-iii/ Spinraza - https://nyemetoder.no/metoder/nusinersen-spinraza Zolgensma - https://nyemetoder.no/metoder/onasemnogene-abeparvovec-zolgensma Since introduction of newborn screening in Norway most patients is either treated as newborns with gene-therapy Zolgensma (treating the root cause of disease) or Evrysdi/Spinraza. Older patients are treated with Evrysdi or Spinraza. In addition to these disease-modifying treatments, a multidisciplinary approach is required for the management of SMA based on the patient'n status and needs over time. This includes rehabilitation, nutritional and orthopedic assessment and respiratory care [1, 2]. In general, the goals of supportive care are to improve or maintain patient's independency in daily life activities by keeping the motor functions, normalizing gas exchange, improving sleep quality, facilitating home care, reducing hospitalizations and intensive care unit admissions,

	and reducing the burden of illness on the patient and their family and/or caregivers [3, 2].
10.5 Prognose *Beskriv prognosen med nåværende behandlingstilbud, inkl. referanse*	Spinal muscular atrophy (SMA) is a rare, genetic, neuromuscular disease characterized by progressive, irreversible motor neuron loss that results in muscle weakness, atrophy, and paralysis, leading to profound physical disability, impairment of swallowing and breathing, and in severe cases, premature death, if not treated [4, 5). In general, patients presenting with symptoms early in life experience more rapid and severe symptom onset, but all patients have a declining clinical course, if untreated [6, 7, 8]. In Norway, newborn screening for SMA was introduced in 2021, and most patients born today receive early treatment. Notably, prior to the availability of DMTs, infantile-onset (non-sitter) SMA was the most common genetic cause of infant mortality, typically resulting in death before the age of 2 years [6]. The introduction of DMTs in recent years have revolutionized the management of SMA and significantly improved disease outcomes, including survival in patients with early onset SMA [9, 10-13]. Newborn screening has improved disease outcomes even more. Nonetheless, there remains a need for therapeutic options that can help a broad range of patients with SMA maintain or improve functional abilities regardless of age, ambulatory status, motor function, or treatment history, without the burden of lifelong administration. Prior to the development and availability of DMTs, SMA was historically classified as five discrete clinical types (Type 0 through Type 4) based on the age of symptom onset and highest motor milestone achievement . [5, 9, 14]. However, current understanding suggests that the availability of DMTs has altered the disease trajectory of SMA and led to the emergence of a broad continuum of functional phenotypes with overlapping symptoms and outcomes across these historical SMA types. Individuals now achieve more motor milestones and functional abilities than predicted by the historical classification, especially if treatment is started early, in the pre-symptomatic phase (for example, if identified via newborn screening) [15-19]. As a result, clinicians now describe patients according to their current functional ability, and clinical guidelines provide recommendations on the management of SMA based on the current functional status of the patient (non-sitters, sitters, walkers) [20, 21,22].

10.6 Det nye legemiddelets innplassering i behandlingsalgoritmen	Equal access on same terms as existing treatments, informed shared-decision-making where all treatments are available. Existing treatments have start/stop (and switch) criterias. There should be updated switch criteria established at launch.
10.7 Pasientgrunnlag *Beskrivelse, insidens og prevalens av pasienter omfattet av aktuell indikasjon* i Norge, inkl. referanse.* *Antall norske pasienter antatt aktuelle for behandling med legemiddelet til denne indikasjonen.* ** Hele pasientgruppen som omfattes av aktuell indikasjon skal beskrives*	Globally, it has previously been estimated that between 1 and 2 people per 100,000 inhabitants have SMA. In Norway, there are approximately 60 individuals under the age of 18 with SMA and around 80 individuals over 18. The number of people living with SMA at any given time is expected to increase following the establishment of specific disease-modifying treatment.https://www.helsenorge.no/sykdom/sjeldne-diagnoser/spinal-muskelatrofi/ According to ID2024_042 there is 83 adult patients with type I-III per dec. 2024. Registry has an assumed coverage of >90 % for SMA. An average of 5 - 7 new cases of spinal muscular atrophy (SMA) are diagnosed in Norway each year, which corresponds to approximately 1 in 11,000 live births. The annual incidence in Europe is approximately 10 to 20 per 100,000 live births [14, 18]. For Norway the incidence rate is between 3,7 (23)– 4.42 [24]). The target population of onasemnogene abeparvovec intrathecal (IT) includes all patients with 5q SMA, but is restricted to those aged 2 years and older. Patients treated with gene therapy for SMA at birth will not be eligible for subsequent treatment with onasemnogene abeparvovec IT, thus, the number of patients eligible for treatment with onasemnogene abeparvovec IT will be significantly lower than the prevalence and incidence rates presented here.

11 Studiekarakteristika for relevante kliniske studier			
	Studie 1	Studie 2	Studie 3
11.1 Studie-ID *Studienavn, NCT-nummer, hyperlenke*	STRONG AVXS-101-CL102 (CL-102) NCT03381729 Study Details | NCT03381729 | Study of Intrathecal Administration of Onasemnogene Abeparvovec-xioi for Spinal Muscular Atrophy | ClinicalTrials.gov	STEER COAV101B12302 (B12302) NCT05089656 Study Details | NCT05089656 | Efficacy and Safety of Intrathecal OAV101 (AVXS-101) in Pediatric Patients With Type 2 Spinal Muscular Atrophy (SMA) | ClinicalTrials.gov	STRENGTH COAV101B12302 (B12302) NCT05386680 Study Details | NCT05386680 | Phase IIIb, Open-label, Multi-center Study to Evaluate Safety, Tolerability and Efficacy of OAV101 Administered Intrathecally to Participants With SMA Who Discontinued Treatment With Nusinersen or Risdiplam | ClinicalTrials.gov
11.2 Studietype og -design	Phase I, open-label, ascending-dose, multi-center trial	Phase III, randomized, sham-controlled, double-blind multi-center study	Phase IIIb open-label, single arm, multi-center study
11.3 Formål	To assess the safety, tolerability, and efficacy of OAV101B	To evaluate efficacy and safety of OAV101B in treatment-naïve patients	To evaluate safety, tolerability and efficacy of OAV101B in patinents that were previously treated with SMN increasing medicines
11.4 Populasjon *Viktige inklusjons- og eksklusjonskriterier*	Patients with SMA and three copies of *SMN2* gene, aged 6 to <60 months, treatment-naïve, able to sit independently	Patients with SMA aged 2 to <18 years, treatment-naïve, able to sit independently but never ambulatory	Patients with SMA aged 2 to <18 years after discontinuation of nusinersen or risdiplam, able to sit independently but never ambulatory
11.5 Intervensjon (n)	A single intrathecal injection of OAV101B	A single intrathecal injection of OAV101B	A single intrathecal injection of OAV101B

Dosering, doseringsintervall, behandlingsvarighet	Cohort 1, OAV101B 6,0E-13 vg, 6 to <24 months: n=3 Cohort 2, OAV101B 1,2E-14 vg, 6 to <24 months: n=13 24 to <60 months:n=12 Cohort 3, OAV101B 2,4E-14 vg, 6 to <24 months: n=4	OAV101B 1,2E-14 vg: n=75 Sham: n=51	OAV101B 1,2E-14 vg: n=27
11.6 Komparator (n) *Dosering, doseringsintervall, behandlingsvarighet*	A population-matched cohort from the Pediatric Neuromuscular Clinical Research (PNCR) natural history data set	Sham control included a small needle prick on the lower back, also under sedation. Sham group received placebo instead of prednisolone with same administration protocol as the treatment arm.	N/A
11.7 Endepunkter *Primære, sekundære og eksplorative endepunkter, herunder definisjon, målemetode og ev. tidspunkt for måling*	Primary endpoint (Safety) To characterize safety and tolerability of OAV101B during the study. Primary efficacy endpoint: Participants 6 to <24 months at time of dosing: Proportion of participants achieving the ability to stand without support for at least 3 seconds (Bayley	Primary endpoint (Efficacy): Change from baseline in HFMSE total score at the end of Period 1 (Week 52) in the overall study population (2 to <18 years age group) in OAV101B vs sham. Secondary efficacy endpoints at 52 weeks: • Achievement of at least a 3-point improvement from baseline in HFMSE total	Primary endpoint (safety): To characterize the safety and tolerability of OAV101B over a 52-week period in patients with SMA aged 2 to <18 years who have discontinued treatment with nusinersen or risdiplam Secondary endpoint efficacy (motor function):

	Scales of Infant and Toddler Development – Gross Motor Subtest Item #40) Participants 24 to <60 months at time of dosing: Change from baseline in HFMSE total score.	score in the overall study population (2 to <18 years age group) • Change from baseline in RULM in the 2 to <18 years age group in OAV101B vs sham • Achievement of at least a 3-point improvement from baseline in HFMSE total score in the 2 to <5 years age group • Change from baseline in HFMSE total score at in the 2 to <5 years age group OAV101B vs sham • Change from baseline in the RULM at the end of Period 1 in the 2 to <5 years age group in OAV101B vs sham Exploratory efficacy outcomes: for the 5 to <18 years age subgroup, change from baseline in HFMSE score and change from baseline in RULM score vs sham at 52 weeks.	• Change from baseline to week 52 visit in HFMSE • Change from baseline to week 52 visit in RULM Secondary objectives also included the change in caregiver experience from baseline to week 52, which was assessed using the ACEND instrument. An exploratory motor objective included assessment of demonstration of motor milestones according to the Developmental Milestone Checklist containing items from the WHO MGRS study
11.8 Relevante subgruppeanalyser *Beskrivelse av ev. subgruppeanalyser*	N/A	N/A	N/A
11.9 Oppfølgingstid *Hvis pågående studie, angi oppfølgingstid for data som forventes å være tilgjengelige for vurderingen hos Direktoratet for medisinske*	Cohorts 1 and 2: 12 months Cohort 3: 15 months	Sham controlled period was 52 weeks (Period 1). Total study duration was 64 weeks. After this patients can continue in the long-term follow-up study.	52 weeks After this patients can continue in the long-term follow-up study.

produkter samt den forventede/planlagte samlede oppfølgingstid for studien			
11.10 Tidsperspektiv resultater *Pågående eller avsluttet studie? Tilgjengelige og fremtidige datakutt*	The follow-up for up to 15 years from dosing continues in long-term follow-up study. AVXS-101-LT002 (LT-002), NCT04042025 Study Details | NCT04042025 | Long-term Follow-up Study of Patients Receiving Onasemnogene Abeparvovec-xioi | ClinicalTrials.gov 18 participants had received the therapeutic dose (4 participants aged 24 to <60 months and 8 participants aged 6 to <24 months) Main efficacy outcome is change from baseline in HFMSE total score. The latest interim data cutoff was in June 2024, and next one is expected to be published in the first half of 2026.	Long-term follow-up study for up to 5 years from enrollment in the follow-up study. COAV101A12308 (A12308) NCT05335876 Study Details | NCT05335876 | Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 in Clinical Trials | ClinicalTrials.gov	Long-term follow-up study for up to 5 years from enrollment in the follow-up study. COAV101A12308 (A12308) NCT05335876 Study Details | NCT05335876 | Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 in Clinical Trials | ClinicalTrials.gov
11.11 Publikasjoner *Tittel, forfatter, tidsskrift og årstall. Ev. forventet tidspunkt for publikasjon*	STRONG-study: Finkel RS, Darras BT, Mendell JR, et al. Intrathecal Onasemnogene Abeparvovec for Sitting, Nonambulatory Patients with Spinal Muscular Atrophy: Phase I Ascending-Dose Study (STRONG). J Neuromuscul Dis.	STEER-study: Proud CM, Vũ DC, Wilmshurst JM, et al. Intrathecal onasemnogene abeparvovec in treatment-naive patients with spinal muscular atrophy: a phase 3, randomized controlled trial. Nat Med. 2025 Dec 8. doi:	STRENGTH-study: Kwon JM, Munell F, Le Goff L, et al. Intrathecal onasemnogene abeparvovec for treatment-experienced patients with spinal muscular atrophy: a phase 3b, open-label trial. Nat Med. 2025 Dec 8. doi: 10.1038/s41591-025-04119-2.

	2023;10(3):389-404. doi: 10.3233/JND-221560 Latest data from the long-term follow-up study LT-002 was presented by Darras BT, et al., (P179) at the 29th Annual Congress of the World Muscle Society (WMS), October 8–12, 2024, Prague, Czechia. The poster reported outcomes with median follow-up time of 5,3 years (min-max, 2,9-6,4) since dosing.	10.1038/s41591-025-04103-w.	

12 Igangsatte og planlagte studier	
12.1 Er det pågående eller planlagte studier for legemiddelet innenfor samme indikasjon som kan gi ytterligere informasjon i fremtiden? *Hvis ja, oppgi forventet tidspunkt*	Ja ☒ Nei ☐ Phase 4 study STREAM is a prospective observational study reflecting US clinical practice. The study will include patients of 18 years of age or older, and either ambulatory or non-ambulatory, or ambulatory patients between 2 and <18 years of age. The study is planned to start later this year.
12.2 Er det pågående eller planlagte studier for legemiddelet for andre indikasjoner?	Ja ☐ Nei ☒ Klikk eller trykk her for å skrive inn tekst.

13 Diagnostikk	
13.1 Vil bruk av legemiddelet til anmodet indikasjon kreve diagnostisk test for analyse av biomarkør? *Hvis ja, fyll ut de neste spørsmålene*	Ja ☒ Nei ☐

13.2 Er testen etablert i klinisk praksis? *Hvis ja, testes pasientene rutinemessig i dag?*	Ja ☒ Nei ☐ Hvis ja, testes pasientene rutinemessig i dag? Ja ☒ Nei ☐
13.3 Hvis det er behov for en test som ikke er etablert i klinisk praksis, beskriv behovet inkludert antatte kostnader/ressursbruk	Klikk eller trykk her for å skrive inn tekst.

14 Andre relevante opplysninger

14.1 Har dere vært i kontakt med fagpersoner (for eksempel klinikere) ved norske helseforetak om dette legemiddelet/indikasjonen? *Hvis ja, hvem har dere vært i kontakt med og hva har de bidratt med?* *(Relevant informasjon i forbindelse med rekruttering av fageksperter i Nye metoder)*	Ja ☒ Nei ☐ We have been in dialogue with Sean Wallace (as the treating physician), he has contributed with clinical insights. He has not received any payment. We have had one meeting with Ingrid Voktor Svinvik on SMA in Norway.
14.2 Anser leverandør at det kan være spesielle forhold ved dette legemiddelet som gjør at en innkjøpsavtale ikke kan basere seg på flat rabatt for at legemiddelet skal kunne oppfylle prioriteringskriteriene? *Hvis ja, begrunn kort.* *Hvis ja, skal eget skjema fylles ut og sendes til Sykehusinnkjøp HF samtidig med at dokumentasjon til metodevurdering sendes til Direktoratet for medisinske produkter.* *Nærmere informasjon og skjema:* Informasjon og opplæring - Sykehusinnkjøp HF	Ja ☒ Nei ☐ This is a rare disease where documentation is scarcer than more common diseases. For reduction of risk and risk sharing it can be beneficial with an alternative price agreement. The other treatments established for SMA in Norway have alternative price agreements.
14.3 Andre relevante opplysninger?	References used in this document: 1. Mercuri, E., et al., Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care. Neuromuscul Disord, 2018. 28(2): p. 103-115

	2. The Society for Neuropediatrics, S2k-Leitlinie Diagnostik und Therapie der 5qassoziierten spinalen Muskelatrophie im Kindes- und Erwachsenenalter. 2024: AWMF Online, . p. 1-110. 3. Yuan, P. and L. Jiang, Clinical characteristics of three subtypes of spinal muscular atrophy in children. Brain Dev, 2015. 37(5): p. 537-41. 4. Kolb, S.J. and J.T. Kissel, Spinal muscular atrophy: a timely review. Arch Neurol, 2011. 68(8): p. 979-844. 5. Kolb, S.J. and J.T. Kissel, Spinal Muscular Atrophy. Neurol Clin, 2015. 33(4): p. 831-46. 6. Kolb, S.J., et al., Natural history of infantile-onset spinal muscular atrophy. Ann Neurol, 2017. 82(6): p. 883-891. 7. Burr, P. and A.K.R. Reddivari, Spinal Muscle Atrophy. 2023, StatPearls [Internet]: Treasure Island (FL): StatPearls Publishing 8. Kaufmann, P., et al., Observational study of spinal muscular atrophy type 2 and 3: functional outcomes over 1 year. Arch Neurol, 2011. 68(6): p. 779-86. 9. Schroth, M., et al., Spinal Muscular Atrophy Update in Best Practices: Recommendations for Diagnosis Considerations. Neurology Clinical Practice, 2024. 14(4): p. e200310. 10. Baranello, G., et al., Prognostic Factors and Treatment-Effect Modifiers in Spinal Muscular Atrophy. Clin Pharmacol Ther, 2021. 110(6): p. 1435-1454. 11. Schroth, M.K., et al., Spinal Muscular Atrophy Update in Best Practices: Recommendations for Treatment Considerations. Neurology Clinical Practice, 2025. 15(1): p. e200374. 12. Pera, M.C., et al., Type I spinal muscular atrophy and disease modifying treatments: a nationwide study in children born since 2016. EClinicalMedicine, 2024. 78: p. 102967. 13. Finnegan, R., et al., Mortality of Symptomatic children with spinal muscular atrophy in the era of disease-modifying therapies. Neuromuscular Disorders, 2025: p. 105313 14. Verhaart, I.E.C., et al., Prevalence, incidence and carrier frequency of 5q-linked spinal muscular atrophy - a literature review. Orphanet J Rare Dis, 2017. 12(1): p. 124 15. Schorling, D.C., A. Pechmann, and J. Kirschner, Advances in Treatment of Spinal Muscular Atrophy - New Phenotypes, New Challenges, New Implications for Care. J Neuromuscul Dis, 2020. 7(1): p. 1-13. 16. Prior TW., Spinal muscular atrophy. 2024.

	17. Mercuri, E., Spinal muscular atrophy: from rags to riches. Neuromuscul Disord, 2021. 31(10): p. 998-1003. 18. Mercuri, E., et al., Spinal muscular atrophy — insights and challenges in the treatment era. Nature Reviews Neurology, 2020. 16(12): p. 706-715. 19. Mercuri, E., et al., Spinal muscular atrophy. Nat Rev Dis Primers, 2022. 8(1): p. 52. 20. Wirth, B., et al., Twenty-Five Years of Spinal Muscular Atrophy Research: From Phenotype to Genotype to Therapy, and What Comes Next. Annu Rev Genomics Hum Genet, 2020. 21: p. 231-61 21. Eggermann, K., et al., Spinal muscular atrophy (5qSMA): best practice of diagnostics, newborn screening and therapy. Medizinische Genetik, 2020. 32(3): p. 263-272. 22. Mercuri, E., et al., Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care. Neuromuscul Disord, 2018. 28(2): p. 103-115 23. Müller, K.I., et al., The prevalence of hereditary neuromuscular disorders in Northern Norway. Brain Behav, 2021. 11(1): p. e01948. 24. Husebye, S.A., et al., A hospital based epidemiological study of genetically determined muscle disease in south western Norway. Neuromuscul Disord, 2020. 30(3): p. 181-185

Informasjon om Nye metoder finnes på nettsiden nyemetoder.no