NYE METODER

v. 20.12.2024

Nye metoder: Innspill til metoder, oppdrag og beslutninger

Bruk dette skjemaet for å gi innspill til metoder i Nye metoder uansett hvor de befinner seg i prosessen. Skjemaet skal for eksempel brukes hvis du har innspill til en metode i en anmodning eller et forslag som skal behandles i Bestillerforum for nye metoder. Det skal også brukes for innspill til oppdrag som er gitt av Bestillerforum, og for innspill til beslutninger som er tatt.

Det er generelt ønskelig at innspill kommer inn så tidlig som mulig i prosessen, gjerne før metoden behandles i Bestillerforum.

Utfylt skjema sendes til Sekretariatet for Nye metoder; nyemetoder@helse-sorost.no. Merk e-posten med "innspill" og ID-nummer.

Merk: **Punkt 1-3 og 11 skal fylles ut av alle.** Øvrige punkter fylles ut avhengig av hva innspillet gjelder.

Jeg er klar over at skjemaet kan bli publisert i sin helhet på nyemetoder.no (kryss av): ☒
Har du informasjon du mener ikke kan offentliggjøres, ta kontakt med sekretariatet før innsending.

Jeg har fylt ut punkt 11 «Interesser og eventuelle interessekonflikter» (kryss av): ☒

1.Hvilken metode gjelder innspillet?	
ID-nummer*:	ID2018_115
Metodens navn:	Lomitapid (Lojuxta)

*ID-nummer finner du på metodesiden på nyemetoder.no og har formen ID2024_XXX.

2. Opplysninger om den som gir innspill	
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3. Innspill til metode, oppdrag, eller beslutning (besvares av alle)
Skriv kort og oppsummer gjerne hovedpoenget.

Note on new evidence since the initial assessment of Lojuxta
Lojuxta was initially discussed in Beslutningsforum in 2018 and the latest decision was in 2023. Since then, there has been evidence published which we describe new/important information in this note.

Summary
In summary, there is extensive long-term real-world evidence of the consequences of Lojuxta treatment available, which confirms the results seen in the clinical trial setting. Multiple real-world studies demonstrate lomitapide's ability to enable patients to achieve and maintain LDL-C targets with over 9 years of data. Multiple studies also demonstrate that up to 80% of

patients are able to stop lipoprotein apheresis (LA) while maintaining LDL-C levels without increasing doses of concomitant lipid-lowering-therapies.

Effectiveness
Multiple real-world studies are available. A recent literature review published in March 2025 reviewed existing clinical trial and RWE data on efficacy and safety, concluded that lomitapide is supported by strong evidence as an effective LDLR-independent therapy for reducing LDL-C and non-HDL-C levels in patients with HoFH and that lomitapide demonstrates an acceptable safety profile for long-term use alongside other available lipid-lowering therapies (Arca 2025).
Among the individual studies available, D'Erasmo and colleagues assessed changes in long-term LDL-C burden and goals achievement in two independent HoFH patients' cohorts, one treated with lomitapide in Italy (n=30) and the other with LA in France (n=29). The adjunct of lomitapide to conventional lipid-lowering therapies determined an additional 58.0% reduction of last visit LDL-C levels, compared to 37.1% when LA was added (D'Erasmo 2021).
Results from another, multi-centre, retrospective, observational study including 75 HoFH patients treated with lomitapide in a real-world clinical setting from 9 European countries were published in 2021. LDL-C changes, AEs, and MACE were assessed. After a median 19 months (inter-quartile range 11–41 months) of treatment with a median dosage of 20 mg of lomitapide, LDL-C decreased by 56%. Among 38 patients with HoFH treated with LA at baseline, 50% discontinued LA after lomitapide initiation. (D'Erasmo 2022).
LOWER is a global, prospective, observational cohort study of the long-term safety and effectiveness of lomitapide in clinical practice across sites in the US, Europe, Canada, Argentina and Taiwan. Enrolment began in March 2014 and data collection will continue for 10 years from enrolment (Blom 2024, Underberg 2020). As of February 2023, 226 patients (43.4% male) were enrolled in LOWER. Post-lomitapide data were available for 223 patients (98.7%); mean lomitapide exposure was 38.5 months. Lomitapide doses ranged from 2.5–50 mg/day and the global mean dose was 13.0 mg/day (17.4 mg/day in patients treated in Europe). Sixty-eight (68.6%) of patients enrolled in LOWER experienced a reduction in LDL-C by ≥50% from baseline.
In another study by Kolovou et al, lomitapide was added in 12 homozygous familial hypercholesterolaemia patients treated with standard lipid-lowering drugs +/- biweekly lipoprotein apheresis sessions (nine patients). The follow-up period with lomitapide treatment was 3–24 months (13.8 +/- 7.9). The addition of lomitapide lowered LDL-C levels further by 56.8% compared to lipid-lowering drugs alone, and by 54% comparing to lipid-lowering drugs to lipoprotein apheresis (Kolovou 2020).
A publication from Italy described the real-world usage of lomitapide (D'Erasmo 2017) in 15 patients, 10 of whom were receiving regular apheresis. Eigth of the 10 patients (80%) stopped apheresis after they had started taking lomitapide and maintained significant LDL-C lowering with a mean LDL-C at target. The same was true in a cohort in Greece where 78% of patients discontinued apheresis after they started taking lomitapide and the remaining 22% reduced apheresis frequency to once a month (Kolovou et al 2020). In another, Pan-European study, LDL apheresis (LA) was stopped 13 patients (17%) when their respective nadir LDL-C values were obtained and another 14 patients (38%) stopped at last follow-up (D'Erasmo 2022).

Tolerability
Experience and published data on the real-world use of lomitapide in HoFH has not shown any unanticipated safety issues and suggests that both GI tolerability and effects on the liver can be managed in the real-world setting (D'Erasmo 2017; deGoma 2014; Kolovou 2016; Stefanutti 2016; van Lennep 2015, Blom 2024, Underberg 2020, Larrey 2022, D'Erasmo 2022). In the analysis of long-term 9-year data from over 226 patients from LOWER study, no new hepatic safety signals were identified compared to the original adult clinical study and in patients who

had hepatic enzyme elevations, temporary interruption of lomitapide or reduction of the dose resulted in resolution of the elevation and allowed treatment to continue (Underberg 2020, Blom 2024).

Avoided burden of alternative treatment options
Apheresis greatly impacts a patient's quality of life. In two studies, it has been shown that the impact of apheresis on a patient's quality of life is the same as haemodialysis (Stasiewski, 2015; Roasada, 2016). Another study reported almost 70% of HoFH patients who have regular apheresis "*considered the apheresis sessions to be too tiring, painful/uncomfortable, time-consuming and incompatible with full-time employment*." (Bruckert 2014). In addition, 60% of HoFH patients found it "*Time-consuming, cannot catch up with school, work, and family*" and 64% reported "*Challenges related to procedure, pain, needles etc"* (Kayikcioglu 2019).
Wyld et al (Wyld 2012) in a systematic review and meta-analysis reported a utility decline associated with dialysis of 0.11. This data may be used to estimate a utility decline of all other intravenous/hospital-based treatment options in HoFH.

References
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Mer detaljert informasjon og innspill til PICO*

*PICO er et verktøy for å formulere presise problemstillinger i metodevurderingsarbeid. PICO er en forkortelse for Population/Problem – Intervention – Comparison – Outcome. PICO brukes til å presisere hvilken populasjon/problem som skal studeres, hvilke(t) tiltak (metode/behandling) som skal vurderes, hvilket tiltak det er naturlig å sammenligne med, og hvilke utfall/endepunkter det å er relevant å måle/vurdere. PICO er viktig for planlegging og gjennomføring av en metodevurdering.

4. Kjenner du til om metoden er i bruk i Norge i dag?

Er metoden i bruk utenom kliniske studier i dag (kryss av hvis ja): ☐
Fra hvilket tidspunkt har den vært i bruk: Klikk eller trykk her for å skrive inn tekst.
Hvor er eventuelt metoden i bruk: Klikk eller trykk her for å skrive inn tekst.

5. Hvilken pasientgruppe i den norske spesialisthelsetjenesten er metoden aktuell for? (PICO)

Beskriv kortfattet: Klikk eller trykk her for å skrive inn tekst.

6. Er du kjent med behandlingsalternativer til denne metoden og hvordan disse fungerer for pasientgruppen i dag? (PICO)

Beskriv kortfattet: Klikk eller trykk her for å skrive inn tekst.

7. Har du innspill til hva som vil være viktig for pasienter som er aktuelle for behandling med metoden? (PICO)
Hva kan oppfattes som en fordel for pasienter og brukere med denne metoden sammenlignet med aktuelle alternativer? Hvilke endepunkter/resultater av behandlingen er det aktuelt å måle? Beskriv kortfattet: Klikk eller trykk her for å skrive inn tekst.

8. Spesielt for medisinsk utstyr (besvares av leverandør): CE-merking
Foreligger det CE-merking for bruksområdet som beskrives i metoden? I så fall angi type og tidspunkt:Klikk eller trykk her for å skrive inn tekst.

9. Spesielt for legemidler (besvares av leverandør): Markedsføringstillatelse (MT)
Har legemiddelet MT for indikasjonen som omfattes av metoden? Angi i så fall tidspunkt eller ventet tidspunkt for MT: Klikk eller trykk her for å skrive inn tekst.

10. Andre kommentarer
Klikk eller trykk her for å skrive inn tekst.

11. Interesser og eventuelle interessekonflikter Beskriv dine relasjoner eller aktiviteter som kan påvirke, påvirkes av eller oppfattes av andre å ha betydning for den videre håndteringen av metoden som det gis innspill på (for eksempel: økonomiske interesser i saken, oppdrag eller andre bindinger).
Beskriv kortfattet: Chiesi Pharma AB är tillverkare av produkten