**NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE**

CENTRE FOR HEALTH TECHNOLOGY EVALUATION

Highly Specialised Technologies

Consultation on Batch 63 draft remits and draft scopes and

summary of comments and discussions at scoping workshops

# Topic list

## Topic ID: 1473

Topic title: Onasemnogene abeparvovec for treating spinal muscular atrophy type 1.

# Provisional Title: Onasemnogene abeparvovec treating spinal muscular atrophy type 1.

**Topic Selection ID Number:** 9395.

**HST ID Number:** 1473.

**Company:** Avexis.

**Anticipated licensing information:** \*\*\*Confidential information removed\*\*\*

## Draft Remit

To appraise the clinical and cost effectiveness of onasemnogene abeparvovec within its marketing authorisation for treating spinal muscular atrophy type 1.

## Main points from consultation

Following the consultation exercise and the scoping workshop, NICE is of the opinion that a **HST** evaluation of onasemnogene abeparvovec for treating spinal muscular atrophy type 1 is appropriate.

The proposed remit is **not appropriate** and should be amended in line with standard wording for **HST**.

All of the HST criteria are met for this indication. At DP3 there was uncertainty around 4 HST criteria; small population size, whether SMA 1 was a distinct clinical group, treatment of SMA in highly specialised services and whether the treatment had the potential for life long use. Following the consultation and scoping workshop we are of the opinion that these criteria have now been met:

* In 2017 the incident population of SMA type 1 was 40 (prevalent population estimated to be 40 or less)
* Type 1 SMA is identified based on defined clinical criteria
* NHS England confirmed this technology will be delivered in 2 or 3 centres as a highly specialised service
* The technology is a gene therapy, expected to be used as a one-off treatment, and the benefits are expected to be lifelong.

## Population Size

Approximately 40 people in England would be eligible for treatment with this technology.  
In 2017 it is estimated that 68 children were born with SMA in England and Wales.

* Estimated that 60% of diagnoses are type 1, implying approx. 40 new diagnoses of type 1 per year

The treatment is indicated only for children up to a maximum of 8.5kg in body weight and due to the severity of the disease the life expectancy for these patients is usually less than 1 year. Therefore the experts at the workshop estimated that the prevalent population is likely to be 40 or less.

## Process (TA/HST): HST.

## Proposed changes to remit

To **evaluate the benefits and costs of** onasemnogene abeparvovec within its marketing authorisation for treating spinal muscular atrophy type 1 **for national commissioning by NHS England.**

## Costing Implications

The unit cost of onasemnogene abeparvovec is unknown so the resource impact of this technology cannot currently be estimated.

## Timeliness statement

Assuming that the anticipated date of the marketing authorisation is the latest date that we are aware of and the expected referral date of this topic, issuing timely guidance for this technology will be possible.