



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

21 November 2018  
EMA/814866/2018  
Inspections, Human Medicines Pharmacovigilance & Committees Division

## Scientific recommendation on classification of advanced therapy medicinal products

Article 17 – Regulation (EC) No 1394/2007

**Disclaimer:** This document is a summary for public release of a scientific recommendation on classification of advanced therapy medicinal products. The original text adopted by the Committee for Advanced Therapies (CAT) has been redacted to delete commercially confidential information.

The present scientific recommendation refers exclusively to the case as presented to the European Medicines Agency (EMA) without prejudice to future evaluations by the Agency.

It is stressed that the scientific recommendation on advanced therapy classification does not amount to any endorsement of the plausibility of the product, including the mode of action or therapeutic indication(s) claimed by the applicant.

### **Brief description (or name when available) of the active substance(s)**

Recombinant adeno-associated virus serotype 9 vector encoding the soluble lysosomal enzyme TPP1.

### **Brief description of the finished product**

Solution for injection.

### **Proposed indication**

Treatment of CLN2 disease.

### **EMA/CAT conclusion**

The procedure was finalised on 18 October 2018 for the following recommendation.

On the basis that:

- the product contains a biological medicinal product as the active substance;



- the active substance consists of recombinant nucleic acid administered to human beings with a view to adding a genetic sequence;
- its therapeutic effect relates directly to the product of genetic expression of this sequence,

the EMA/CAT considers that the product falls within the definition of a gene therapy, as provided in Article 2(1) of Regulation (EC) 1394/2007.