04.2025

KANUMA
קנומה

SEBELIPASE ALFA
Concentrate for solution for infusion IV

רופא/ה ,רוקח/ת נכבד/ה,

חברת אלקסיון פארמה ישראל בע"מ מבקשת להודיע על עדכון העלון לרופא של התכשיר שבנידון.

ההתוויה הרשומה לתכשיר בישראל:
KANUMA is indicated for long-term enzyme replacement therapy (ERT) in patients of all ages with lysosomal acid lipase (LAL) deficiency.

בהודעה זו מצוינים העדכונים המהותיים שחלו במשטר המינון וצורת המתן של התכשיר בעלון לרופא
מידע שהוסר – מסומן ב~~קו אדום חוצה~~
תוספת – כתב כחול

4.2 Posology and method of administration

KANUMA treatment should be supervised by a healthcare professional experienced in the management of patients with LAL deficiency, other metabolic disorders, or chronic liver diseases. KANUMA should be administered by a trained healthcare professional who can manage medical emergencies.

Posology

It is important to initiate treatment as early as possible after diagnosis of LAL deficiency.

For instructions on the preventive measures and monitoring of hypersensitivity reactions, see section 4.4. Following the occurrence of a hypersensitivity reaction, appropriate pre-treatment should be considered according to the standard of care (see section 4.4).

~~***Infants (< 6 months of age)***~~

~~**The recommended starting dose in infants (< 6 months of age) presenting with rapidly progressive LAL deficiency is 1 mg/kg administered as an intravenous infusion once weekly. Dose escalation to 3 mg/kg once weekly should be considered based on clinical response.**~~

Patients with Rapidly Progressive LAL Deficiency Presenting within the First 6 Months of Life

The recommended starting dose in infants (< 6 months of age) presenting with rapidly progressive LAL deficiency is either 1 mg/kg or 3 mg/kg administered as an intravenous infusion once weekly, depending on the clinical status of the patient. A higher starting dose of 3 mg/kg should be considered based on the severity of the disease and rapid disease progression.

Dose escalation should be considered based on suboptimal response to clinical and biochemical criteria, including, e.g., poor growth (especially mid-upper arm circumference, MUAC), deteriorating biochemical markers (e.g. liver transaminases, ferritin, C-reactive Protein, and coagulation parameters), persistent or worsening organomegaly, increased frequency of intercurrent infections, and persistent worsening of other symptoms (e.g. gastrointestinal symptoms):

- **a dose escalation to 3 mg/kg should be considered in case of suboptimal clinical response;**
- **a further dose escalation up to 5 mg/kg should be considered in case of persistent suboptimal clinical response.**

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Further dose adjustments, as a reduction of the dose or an extension of the dose interval, can be made on an individual basis based on achievement and maintenance of therapeutic goals. Clinical studies evaluated doses ranging from 0.35 ~~1~~ to 5 mg/kg once weekly, with one patient receiving a higher dose of 7.5 mg/kg once weekly. Doses higher than 7.5 mg/kg have not been studied.

~~Children and adults~~ ***Pediatric and Adult Patients with LAL Deficiency***
The recommended dose in children and adults who do not present with rapidly progressive LAL deficiency prior to 6 months of age is 1 mg/kg administered as an intravenous infusion once every other week. **Dose escalation to 3 mg/kg once every other week should be considered based on suboptimal response to clinical biochemical criteria, including; e.g., poor growth persistent or deteriorating biochemical markers (e.g., parameters of liver injury (ALT, AST), parameters of lipid metabolism (TC, LDL-c, HDL-c, TG), persistent or worsening organomegaly, and persistent worsening of other symptoms (e.g., gastrointestinal symptoms).**

Special populations

Renal ***~~or hepatic~~*** *impairment*
No dosing adjustment is recommended in patients with renal ***~~or hepatic~~*** impairment based on current knowledge of the pharmacokinetics and pharmacodynamics of sebelipase alfa (See section 5.2).

Hepatic impairment

No dosing adjustment is recommended in patients with hepatic impairment based on current knowledge of the pharmacokinetics and pharmacodynamics of sebelipase alfa (see section 5.2).

(…)

Method of administration

KANUMA is for intravenous use only.

The total volume of the infusion should be administered over approximately 2 hours. A 1-hour infusion may be considered **for those patients receiving the 1 mg/kg dose** after patient tolerability is established **(For the recommended infusion volumes, see section 6.6.)**. The infusion period may be extended in the event of dose escalation.

(…)

6.6 Special precautions for disposal and other handling

(…)

Table 5: Recommended infusion volumes*

Weight range (kg)	**1 mg/kg dose**	**3 mg/kg dose****	**5 mg/kg dose****
	Total infusion volume (ml)	**Total infusion volume (ml)**	**Total Infusion Volume (ml)**
1-2.9	**4**	**8**	**12**
3-5.9	**6**	**12**	**20**
~~1~~ **6**-10.9	10	25	**50**

11-24.**9**	25	50	**150**
25-49.**9**	50	100	**250**
50-99.**9**	100	250	**500**
100-120.**9**	250	500	**600**

* The infusion volume should be based on the prescribed dose and should be prepared to a final sebelipase alfa concentration of 0.1-1.5 mg/ml.

** **For patients with LAL Deficiency presenting within the first 6 months of life who do not achieve an optimal clinical response with a dose of 3 mg/kg** ~~**For patients who do not achieve an optimal clinical response with a dose of 1 mg/kg**~~.

Any unused medicinal product or waste material should be disposed of in accordance with local requirements.

קיימים בעלון עדכונים נוספים, למידע נוסף יש לעיין בעלון לרופא המעודכן.

העלון נשלח לפרסום במאגר התרופות שבאתר משרד הבריאות, וניתן לקבלו מודפס על ידי פניה לבעל הרישום (אלקסיון פארמה ישראל בע"מ, ת.ד. 7063, פתח תקווה 4917001; טלפון: 03-9373753 ; פקס: 03-9373774)

בברכה,

עוז וולך הרוקח הממונה של בעל הרישום