MEDISON

יולי 2022

רופא/ה נכבד/ה
רוקח/ת נכבד/ה,

הריני להודיעכם כי העלון לרופא של התכשיר עודכן:

ONPATTRO 2 mg/ml **אונפטרו 2 מ"ג /מ"ל**

CONCENTRATE FOR SOLUTION FOR INFUSION

הרכב:

Each mL contains patisiran sodium equivalent to 2 mg patisiran.

התוויה:

Onpattro is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy.

להלן העדכונים בעלון לרופא:

Section 4.2
Special populations
~~*Liver transplant*~~
~~Onpattro has not been studied in patients with prior liver transplant; however, no dose adjustments are considered necessary.~~

Section 4.8
Other special population(s)
Liver transplant recipients
In an open-label study in 23 hATTR amyloidosis patients with polyneuropathy progression post liver transplant, the safety profile of patisiran was consistent with previous clinical studies (see section 5.1).

Section 5.1
Mechanism of action
Onpattro contains patisiran, a double-stranded small interfering ribonucleic acid (siRNA) that specifically targets a genetically conserved sequence in the 3' untranslated region of all *variant* ~~mutant~~ and wild-type TTR mRNA.

Clinical efficacy
Liver transplant recipients
In an open-label study, 23 patients with hATTR amyloidosis and polyneuropathy progression after receiving a liver transplant were treated with patisiran at a dose of 300 micrograms per kg via IV infusion once every 3 weeks. Median time from transplant to first patisiran dose was 9.4 years and median duration of patisiran treatment was 13.1 months. All patients received

Medison Pharma
10 Hashiloach St., P.O.B 7090, Petach-Tikva 4917002 Israel
Tel. +972.3.925.0250, Fax. +972.3.922.5740
medisonpharma.com

concomitant immunosuppressants. The study demonstrated a statistically significant median reduction in serum TTR levels from baseline of 91% ($p < 0.001$). Patients also showed stable or improved efficacy endpoints at Month 12 compared to baseline. This was consistent with the findings in the placebo controlled patisiran study.
~~*Patients also showed mean improvement or stability at Month 12 compared to baseline, in measures of neuropathy impairment (NIS), patient-reported measures of quality of life (Norfolk QoL-DN), autonomic neuropathy symptoms (COMPASS-31), activities of daily living and social participation limitations (R-ODS), and nutritional status (mBMI). This was consistent with the findings in the placebo-controlled patisiran study.*~~

Section 5.2
Special populations
Liver transplant
In a clinical study in hATTR amyloidosis patients who had undergone prior liver transplant, steady state pharmacokinetic parameters and TTR reduction were comparable to those observed in patients without a liver transplant.

העלון לרופא נמצא בקישור וכן מפורסם במאגר התרופות באתר משרד הבריאות וניתן לקבלו מודפס על ידי פניה לבעל הרישום.

בברכה,

שרון עמיר
רוקחת ממונה
מדיסון פארמה בע"מ

Medison Pharma
10 Hashiloach St., P.O.B 7090, Petach-Tikva 4917002 Israel
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