HSD120

Tafamidis in the Treatment of Transthyretin Amyloid Cardiomyopathy in Poland – Epidemiological Data and Demographic Data from an Expanded Access Program

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BACKGROUND:

- Transthyretin amyloid cardiomyopathy (ATTR-CM) is a rare and progressive heart condition characterized by the abnormal buildup of amyloid protein deposits in the myocardium walls. There are two types of ATTR-CM: hereditary and wild-type (1).
- Tafamidis is an effective treatment in ATTR-CM (2).
- Despite advances, ATTR-CM is still underdiagnosed and substantial between-country phenotypic variation due to genetic factors has been reported (3). Hence, understanding the epidemiology and demographic structure for each individual country is needed.

OBJECTIVES:

- We aimed to present information on the epidemiology and demographics of patients diagnosed with ATTR-CM and treated with tafamidis in Poland as part of an expanded access program.

METHODS:

- Tafamidis is currently not approved for reimbursement in Poland.
- Prior to approval, patients with ATTR-CM were treated with tafamidis when requested by treating physicians as part of an expanded access program.
- We collected data on all patients included until April 2023 and reviewed the number of patients overtime and the demographic structure.

Fig. 1. Number of patients enrolled in the expanded access program

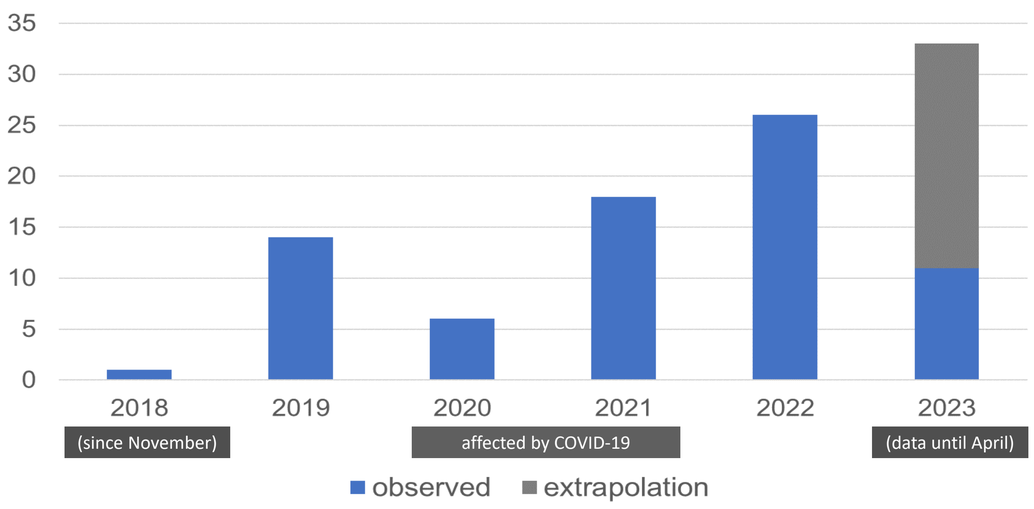
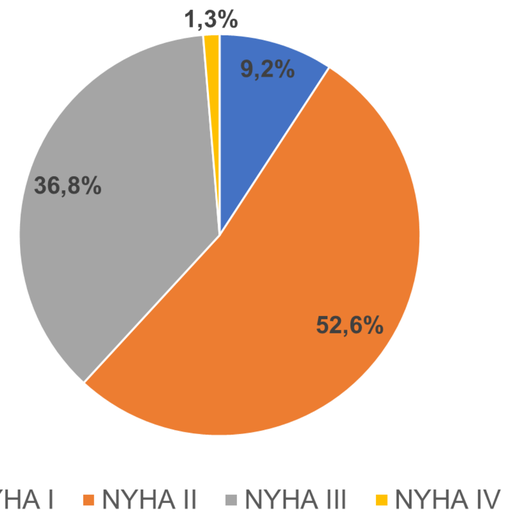


Fig. 2. The structure of NYHA class at enrollment.



RESULTS:

- Altogether, 76 patients were included since November 2018. The number of patients increased over time (with a momentarily decrease during the onset of COVID-19): for 2018-2023, it amounted to, respectively: 1, 14, 6, 18, 26, and 11 (33, after scaling to the whole 2023).
- The patients were mostly men (95.6%), on average71.2 years old (standard deviation = 10.3 years).
- At enrolment, the majority of patients were in NYHA II class (52.65%), 36.8% in NYHA III, 9.2% in NYHA I, and 1.3%in NYHA IV.

CONCLUSIONS:

Expanded access is a pathway for patients with life-threatening conditions to gain access to investigational products for treatment outside of clinical trials when no comparable alternative therapy options are available. Unsolicited requests are submitted by physicians on behalf of patients. ATTR-CM is challenging to diagnose, therefore the number of diagnosed is likely to increase as experience is gained on this condition.

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