

Treatment Landscape for Patients with Advanced Systemic Mastocytosis in Germany in 2015 to 2020 Based on Claims Data Analysis

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Background

- Advanced systemic mastocytosis (AdvSM) is a rare disorder characterized by abnormal growth and accumulation of mast cells which leads to mediator release and/or organ dysfunction/damage with therefore reduced life expectancy.^{1,2}
- Due to the rarity of the disease, only limited data on epidemiology is available.
- Midostaurin was the only drug approved for treatment of AdvSM patients in Germany during the study period. Avapritinib received EMA approval in March 2022 and was therefore not included in the analysis.

Objective

- The study objective was to describe the distribution of systemic therapy prescriptions and prevalence of adult AdvSM patients in Germany based on claims data from 2015 to 2020.

Methods

- A retrospective claims data analysis using German statutory health insurance data from the Institute for Applied Health Research Berlin (InGef) database was performed.
- The analyzed sample of the InGef database (~5% of the German population³) is adjusted by age and gender to represent the German population⁴ and shows good overall accordance with the German population in terms of morbidity, mortality and drug use⁵.
- Adult AdvSM patients (≥ 18 years) were identified by ICD-10-GM codes C94.3 (mast cell leukemia) and C96.2 (malignant mast cell neoplasm) in the most recent available year in the database.
- Systemic therapies between 2015 and 2020 were identified by ATC/OPS codes (following current medical guidelines⁶ and medical expertise) and included antineoplastic agents (ATC code: L01), midostaurin (ATC code: L01XE39 or OPS code: 6-00a.b), cladribine (ATC code: L01BB04), imatinib (ATC code: L01XE01 or OPS code: 6-001.g), hydroxyurea (ATC code: L01XX05), interferon (ATC code: L03AB), and allogeneic stem cell transplant (OPS code: 8-863/5-411).
- Patients with at least one prior systemic therapy identified by the codes above needed to have a recorded ICD-10-GM diagnosis code for AdvSM in the same, following, or preceding quarter as the prescription date.

Results

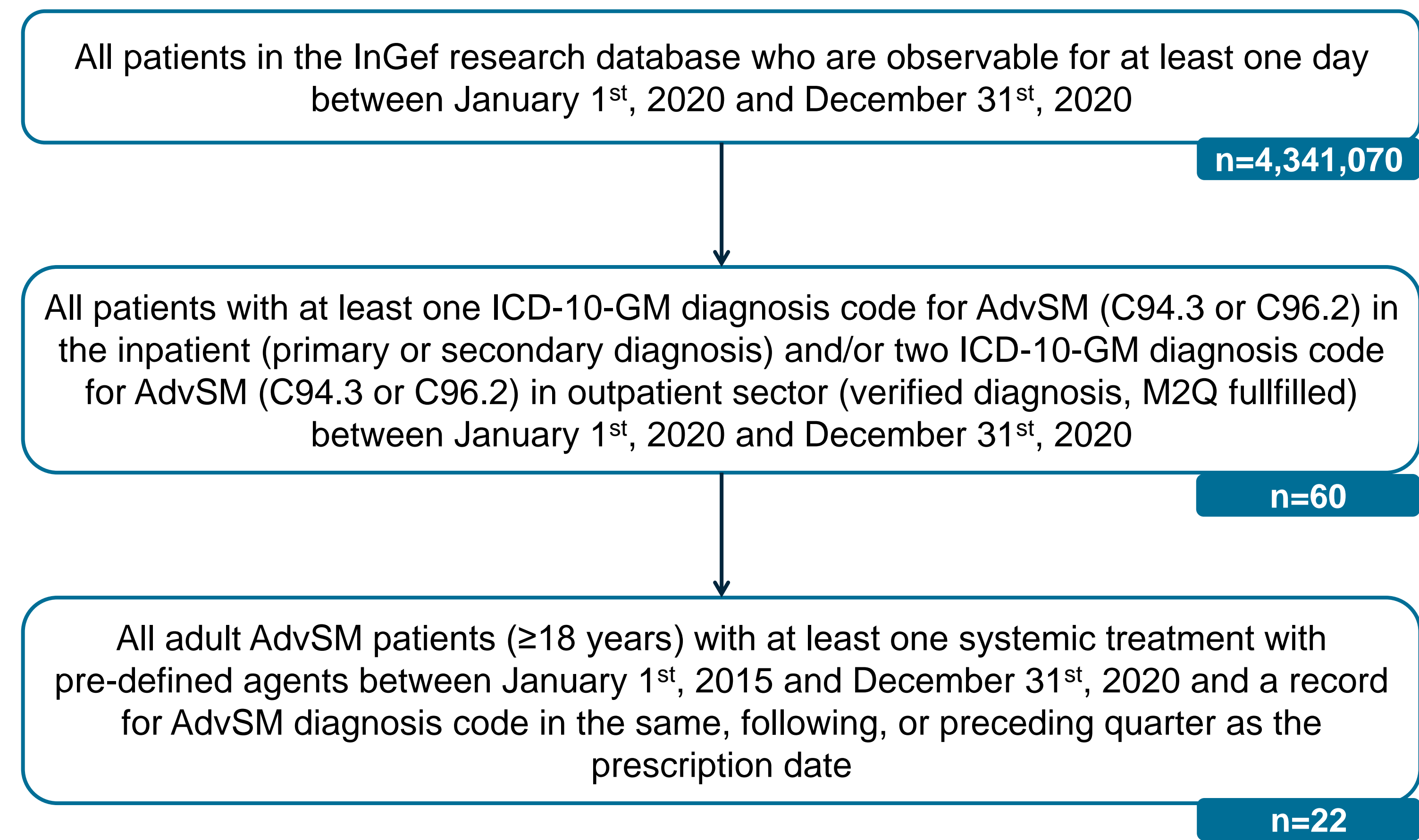
- A total of 22 adult patients had at least one recorded inpatient (primary or secondary diagnosis) ICD-10-GM diagnosis code for AdvSM and/or at least two outpatient recorded diagnosis codes for AdvSM (verified diagnosis) in 2020 as well as at least one systemic treatment with pre-defined agents between 2015 and 2020 (see **Figure 1**).
- This corresponds to the prevalence of 0.51 per 100,000 individuals.
- About 90.9% (n=20) of identified patients had a prescription for antineoplastic agents (see **Figure 2**).
- Midostaurin was prescribed to 27.3% (n=6) of these patients.
- None of the patients underwent allogeneic stem cell transplantation in the study period.

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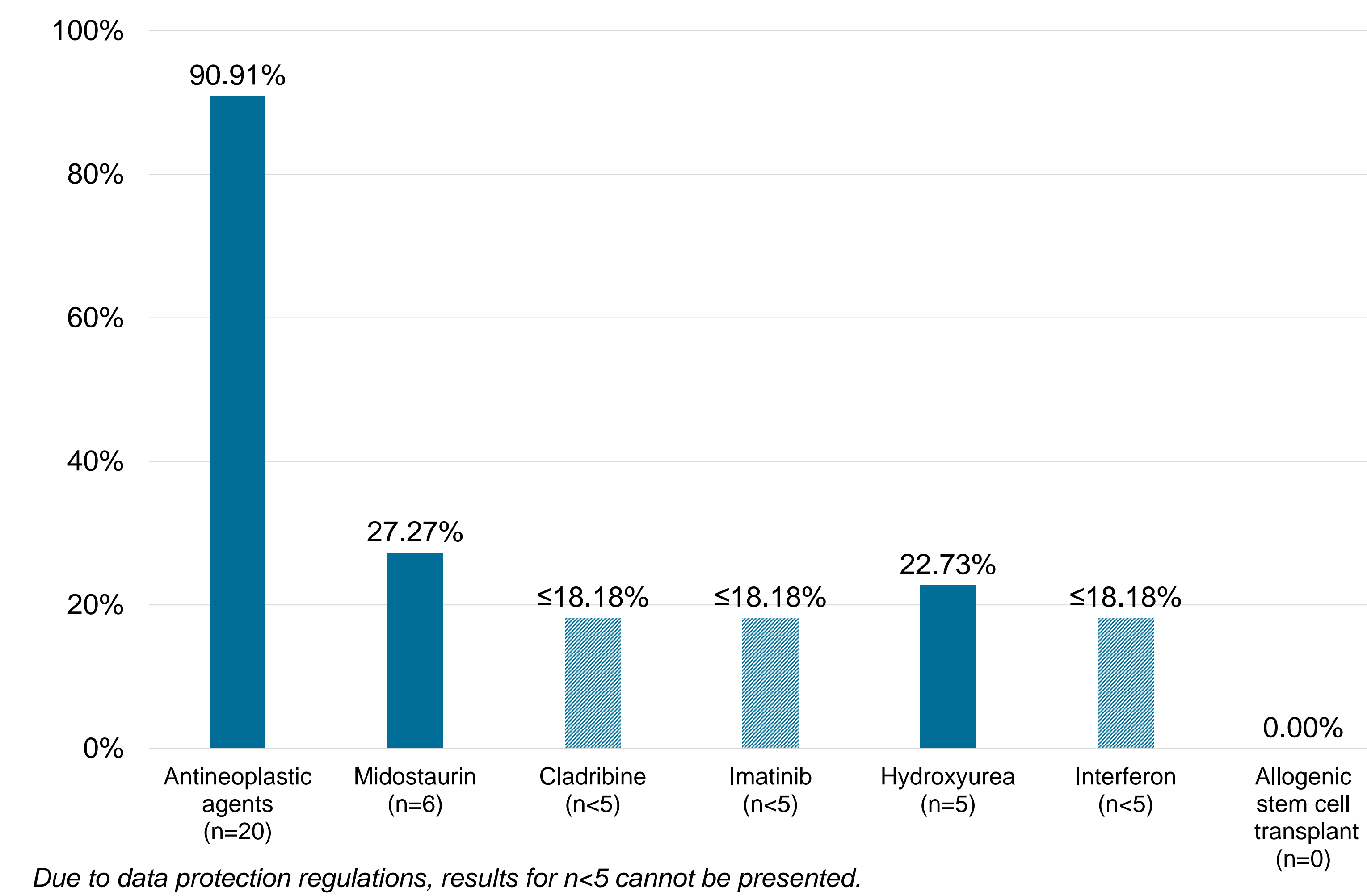
Results (Continued)

Figure 1. Flow-chart of patient selection



Abbreviations: AdvSM, Advanced systemic mastocytosis; ICD-10-GM: International Statistical Classification of Diseases and Related Health Problems, 10th Revision, German Modification; InGef, Institute for Applied Health Research Berlin; M2Q: At least two quarters.

Figure 2. Distribution of systemic therapy prescriptions in adult patients with AdvSM



Due to data protection regulations, results for n<5 cannot be presented.

Discussion

- Our results for the prevalence calculation (0.51 AdvSM cases per 100,000 individuals) are within the range of the recently published study from Schwaab et al. (using data from two reference centers in Germany covering substantial regions in Aachen and Mannheim from 2003 to 2018), in which the calculated prevalence of AdvSM ranged from 0.33 to 0.7 per 100,000 individuals.⁷
- Moreover, the results were consistent with the medical guidelines.
- However, midostaurin, which was the only drug approved for treatment of AdvSM patients in Germany, was prescribed to less than one third of AdvSM patients. This probably indicates the high unmet medical need.