### Background

- Gastrointestinal stromal tumors (GIST) are a rare type of sarcoma, driven by activating mutations encoding tyrosine kinase receptors for c-kit (KIT) and platelet-derived growth factor receptor alpha (PDGFRA).
- The cost impact associated with increasing molecular testing rates is discussed, including the effects of treatment allocation decisions and AIAs.

### Objective

The objective is to assess the potential cost impact of increasing molecular testing rates for GIST patients, including the effects of treatment allocation decisions and AIAs.

### Methods

#### Study design

- A model was developed in Microsoft Excel® to estimate the cost impact associated with increased molecular testing rates in GIST patients for PDGFRA exon 18 and KIT exon 9 mutations, for a hypothetical US health plan with 1 million covered lives, on a 12-month incidence basis. All costs are presented in USD (3).

- The model compared costs based on observed current testing rates at diagnosis to a scenario where 100% of patients are tested. Results determine optimal pharmacy and AE costs in a population not expected to benefit from imatinib treatment (Tables 1 and 2).

- Patient population: Patients with metastatic/irresectable GIST, as well as GIST treated in the advanced setting (adjunctive KIT), with PDGFRA exon 18 or KIT exon 9 mutations, were selected for inclusion, given that treatment allocation will change based on testing.

### Results

- **Base case analysis cost impact**
  - An increase in testing rates to 100% for both mutation types is associated with a potential annual cost increase of $15,213 per million members, or $0.015 per member time (PMPY) (2).
  - Increased costs in the base case are driven by increased dosing and longer progression-free survival (PFS) in exon 9 patients.
  - Inclusion of only PDGFRA exon 18 testing results in a cost saving of $0.008 PMPY due to lower pharmacy costs.
  - For PDGFRA exon 18 and KIT exon 9 molecular testing combined, 10 additional patients need to be tested for one patient to receive optimized treatment.
  - The magnitude of the cost impact associated with increased testing remains small across all plan types.

### Conclusions

- Increased molecular testing in GIST is associated with minimal additional cost and a meaningful increase in the number of patients receiving optimized treatment.
- Estimation to be under $0.02 PMPY, even if KIT exon 9 testing is included in the model.
- Increasing PDGFRA 18 testing alone may even lead to modest cost savings.
- The major driver of estimated cost impact is pharmacy costs, but only a minority is directly due to an increased testing rate.
- Improved treatment can be achieved with a moderate amount of additional testing, suggesting that GIST patients tested for one patient to receive optimized treatment.
- Results suggest that the economic impact associated with PDGFRA exon 18 and KIT exon 9 testing should not be a barrier to increase testing rates in this model.

A model estimating the budget impact associated with introduction of axitinib that incorporates these testing costs is currently being developed.

### Table 1: Overview of the GIST cost of testing model

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Description</th>
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<tbody>
<tr>
<td>Treatment allocation</td>
<td>Based on the clinical trials scenario, for patients with KIT exon 9, this is prior to initiation of therapy, but including a 6.4 month attrition rate. For patients with PDGFRA exon 9, this is prior to initiation of therapy, but including a 6.4 month attrition rate.</td>
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### Table 2: Key model assumptions

- **Assumption:** Baseline costs include up-front costs for testing and all associated costs for up to 842 months. Patients are tested at diagnosis, unless otherwise specified.

### Figure 1: Flow of GIST patients through PDGFRA exon 18 and KIT exon 9 testing

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