**INTRODUCTION**

Sunitinib (SU) is an oral, multitargeted receptor tyrosine kinase inhibitor with antagonistic activity.

**Trial Population**

- **Key inclusion criteria**: Histologically or cytologically diagnosed well-differentiated pancreatic islet cell tumor (World Health Organization [WHO] 2000 classification) 
- **Locally advanced or metastatic disease** with disease progression per Response Evaluation Criteria in Solid Tumors (RECIST), version 1.0 document 
- **Progressive disease** at any time during the study, in the opinion of the investigator. 

**Latest radiological imaging** and a radiology data report from a third independent radiologist were used to classify disease progression. Patients who experienced disease progression were assessed for clinical benefit according to investigator assessment only.

**Endpoints**

- **Baseline and on-trial scans** were evaluated independently according to a two-reader, two-time point, blinded-reader, batch-mode paradigm, by independent third-party radiologists.
- **All patients provided written informed consent.**

**Analysis of Progression-free Survival**

- **Primary analysis** was performed on the intent-to-treat (ITT) population, which included all randomized patients (N = 171), with drug assignment according to initial randomization.
- **Secondary analysis** included all patients with technically adequate scans received on or before the date of last tumor assessment on trial.

**RESULTS**

**Baseline Characteristics**

- **ITT population** included all randomized patients (N = 171), with drug assignment according to initial randomization.
- **ITT population** was the basis for all statistical analyses, and the conclusions are based on the primary analysis.

**Progression-free Survival**

- **Median PFS** in the SU arm was 3.6 months (95% CI: 1.7–7.4, 0.449; HR [95% CI]: 0.44 [0.23, 0.83], 0.0001) compared with 2.5 years (95% CI: 1.3–4.2, 0.1) in the placebo arm.
- **All patients in the SU branch** who had an ECOG PS of 0 in both data sets.

**Table 1.**

<table>
<thead>
<tr>
<th>Endpoint</th>
<th>SU (n=86)</th>
<th>Placebo (n=85)</th>
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<tbody>
<tr>
<td>Median PFS (months)</td>
<td>3.6 (1.7–7.4)</td>
<td>2.5 (1.3–4.2)</td>
</tr>
<tr>
<td>HR (95% CI)</td>
<td>0.44 (0.23, 0.83)</td>
<td>1.00 (0.52, 1.92)</td>
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**Safety**

- **AE profile in the SU arm** was neutropenia (12%), fatigue (10%), and diarrhea (3%).
- **AE profile in the SU arm** of the ITT population.

**CONCLUSIONS**

- **The SU arm showed clinical benefit** of SU patients with progressive, well-differentiated pancreatic NET, and organ-preservation potential of SU in patients with progressive disease.
- **A BCR of 4% for the full population (N=171) is ongoing.**

**ACKNOWLEDGMENTS**

- **Supported by** Pfizer Inc. (NY, USA), and all investigators, organizations, and staff for their contributions.

**REFERENCES**