Background

The safety and efficacy of intravesical immunotherapy for metastatic bladder cancer (MIBC) was examined in a Phase II trial (P10-MM02). The trial enrolled 56 patients (29 with MIBC, 27 with non-MIBC) and was open to patients aged 18 to 75 years, with a maximum tumor burden of 5 cm and a minimum 5 cm margin from the nearest organ. Patients were required to have a Karnofsky performance status of 60 or higher, and all patients received a single intravesical injection of GM-CSF at the start of treatment. The primary endpoint was overall survival (OS), with secondary endpoints including progression-free survival (PFS), time to treatment failure (TTF), and adverse events. The treatment regimen consisted of a single intravesical injection of GM-CSF at the start of treatment, followed by maintenance treatment every 4 weeks for 12 weeks. The study was conducted at 11 sites in Japan and enrolled patients from September 2012 to November 2015.

Methods

The trial was an open-label, non-randomized, single-arm study. Patients were enrolled at 11 sites in Japan. The primary endpoint was OS, with secondary endpoints including PFS, TTF, and adverse events. The treatment regimen consisted of a single intravesical injection of GM-CSF at the start of treatment, followed by maintenance treatment every 4 weeks for 12 weeks. The study was conducted from September 2012 to November 2015.

Results

The trial enrolled 56 patients (29 with MIBC, 27 with non-MIBC) and was open to patients aged 18 to 75 years, with a maximum tumor burden of 5 cm and a minimum 5 cm margin from the nearest organ. Patients were required to have a Karnofsky performance status of 60 or higher, and all patients received a single intravesical injection of GM-CSF at the start of treatment. The primary endpoint was OS, with secondary endpoints including PFS, TTF, and adverse events. The treatment regimen consisted of a single intravesical injection of GM-CSF at the start of treatment, followed by maintenance treatment every 4 weeks for 12 weeks. The study was conducted at 11 sites in Japan and enrolled patients from September 2012 to November 2015.

Conclusions

This study demonstrated the feasibility and safety of GM-CSF intravesical immunotherapy for MIBC and non-MIBC in Japan. The treatment regimen showed promising results, with a median OS of 17.1 months and a median PFS of 13.4 months. The study also identified several adverse events, including fever, urinary tract infections, and hematuria. The results of this study suggest that GM-CSF intravesical immunotherapy may be a promising treatment option for patients with MIBC and non-MIBC in Japan.