

Palifermin Treatment of Mucositis in Transplant Patients Reduces Health Resource Use: Phase III Results

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- Origin of Study** USA
- Type of Study** PHASE III, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY
- Objectives** Determine the effects of palifermin versus placebo on resource use by patients with hematologic malignancies undergoing total body irradiation (TBI) and high-dose chemotherapy with peripheral blood progenitor cell transplantation (PBPC)
- Study Design** Resource use for all statistically significant differences in mean utilization was evaluated, without adjustment for multiple comparisons, for patients with all baseline measures who received at least one dose of study drug during this phase III clinical trial (n = 106 for both placebo and palifermin).
For assessment of patient hospitalizations post transplantation, outpatient hospital visits were excluded (n = 99 and n = 100 for placebo and palifermin treatment, respectively).
- Patients** In all, 212 patients received 12 Gy of TBI with 60 mg/kg of etoposide and 100 mg/kg of cyclophosphamide prior to autologous PBPC.
Patients were randomized (1:1) to receive placebo or palifermin 60 µg/kg/day for 3 consecutive days before TBI and 3 consecutive days after PBPC.
- Observations** Use of palifermin resulted in a statistically significant reduction in hospitalization, analgesic use, and incidence of parenteral feeding:

Mean Resource Use for Patients Receiving Placebo or Palifermin

RESOURCE	PLACEBO	PALIFERMIN	DIFFERENCE	P VALUE
Days of inpatient hospitalization	17.3	15.3	2	0.008
Days of analgesic use	11.8	6.8	5	0.0001
Incidence of parenteral feeding for oral mucositis (number of patients)	46	12	34	< 0.001

- Conclusions** Palifermin significantly reduces the incidence and duration of severe mucositis, resulting in significantly reduced resource use in terms of days of hospitalization, analgesic use, and incidence of parenteral feeding, compared with placebo.
- Discussion** Severe oral mucositis is a frequent complication in patients with hematologic malignancies receiving high-dose chemotherapy and/or TBI with hematopoietic stem cell support. Oral mucositis is associated with significantly worse clinical and economic outcomes in hematologic transplant patients.
Previously, treatment of mucositis has represented an unmet medical need, for which no effective therapy was available. In this phase III trial, recombinant human keratinocyte growth factor (palifermin) was shown, in a paper presented by Spielberger et al at the 39th Annual Meeting of the American Society of Clinical Oncology, to reduce the duration and incidence of severe oral mucositis and its related clinical sequelae.

Palifermin Treatment of Mucositis in Transplant Patients Reduces Health Resource Use

This update on palifermin suggests that it may also significantly reduce health resource utilization.

In this presentation, Emmanouilides et al reported a health resource utilization analysis including patients who received at least one dose of palifermin or placebo. At the daily clinical assessment of patients in the phase III palifermin trial, researchers prospectively collected data on hospitalization, analgesic use, parenteral feeding, intubation, use of intravenous antiinfective agents, and antidiarrheal treatment.

They found that palifermin treatment was associated with significant reductions in analgesic use, days of hospitalization, and occurrence of parenteral feeding. For patients who received palifermin, the mean number of inpatient days was 15.3, compared with 17.3 in the placebo group ($P = 0.008$). Parenteral opioids were used less frequently among palifermin-treated patients (mean of 6.8 days of use vs 11.8 days for placebo, $P = 0.0001$).

Parenteral feeding related to oral mucositis was required in 12% of palifermin-treated patients, versus 43% of placebo-treated patients ($P < 0.001$). Mean number of days of parenteral feeding due to oral mucositis was four times higher in the placebo group (6 vs 1.5 for palifermin treatment, $P < 0.001$).

The researchers also found a trend toward less use of intubation and fewer days of intravenous anti-infective use in the palifermin group, compared with the placebo group.

Taken together, these data suggest palifermin may be effective in treating mucositis and ameliorating mucositis-related symptoms in patients undergoing high-dose chemotherapy. In addition, the resource utilization data suggest a potential cost benefit. The investigators plan to use these data to ascertain how cost-effective palifermin might be in clinical practice.

Key Points

- Palifermin treatment of mucositis may significantly reduce health resource utilization.
- Significant reductions, compared with placebo, were seen in analgesic use, hospitalization, and parenteral feeding.

References

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