



CSF Efficacy and Other Attributes: A Nationally Representative Study of CSF Patients and the Oncologists Who Treated Them

Thomas Orsagh, Kylee J. Heap, Jack R. Gallagher
Clarity Pharma Research, LLC

April 22, 2007

Abstract

The purpose of the present study was to collect data on CSF usage in actual clinical settings. A national probability sample of 131 medical oncologists who prescribe CSFs extracted detailed medical history and treatment information from the records of 527 randomly selected CSF patients. Slightly more than half of the physicians reported some restrictions on their usage of CSFs. The vast majority of CSF usage was for the prevention or treatment of chemotherapy-induced neutropenia (CIN). Almost half of the cancer patients had either breast cancer (31%) or lung cancer (16%). When infection occurred, it was unlikely to have occurred after a CSF administration (in only 16% of CSF administrations). Physician expectations for CSF usage were met in more than 97% of CSF administrations (1,478 out of 1,521 administrations).

Study Background and Objectives

Colony-stimulating factors (CSFs) are hematopoietic growth hormones that stimulate the production, maturation, and function of white blood cells. CSFs have become increasingly important agents in the treatment armamentarium of medical oncologists (Goldwein, et al, *Abramson Cancer Center University of Pennsylvania, OncoLink*, November 1, 2001). Estimates derived from national studies indicate that about 470,000 patients received a CSF in 2006 compared with about 185,000 patients in 1994 (Gallagher et al., *National Retrospective CSF Patient Study of 1994 and of 2006*).

Swanson and associates noted the paucity of research on CSF usage in actual clinical settings and the shortcomings of existing surveys and clinical studies, concluding that the practice patterns of oncologists outside the context of CSF clinical trials remain largely unknown (*Journal of Clinical Oncology*, Vol. 18, Issue 8 (April), 2000: 1764-1770).

The purpose of the present study was to collect data on CSF usage in actual clinical settings using a nationally representative sample of patients treated with CSFs by a nationally representative sample of oncologists.

Methodology

A national probability sample of 131 medical oncologists who prescribe CSFs extracted detailed medical history and treatment information from the records of 527 randomly selected CSF patients. The last four patients treated with a CSF by the physician study participant were selected for the study. Study data were transmitted to researchers by fax or mail. Up to seven follow-up contacts were made with initial non-responding physicians to enhance study participation.

Key Findings of Study

Of the 1,553 CSF administrations to patients in the probability sample, almost 3 out of 4 administrations (73%) were in a physician's office, and 4% were self-administered at home.

- Almost 9 out of 20 physician study participants (44%) reported that they experience "no restrictions" on their prescribing of any CSF.
- More than one-fifth of physicians reported one or more of the following constraints on their prescribing of CSFs:
 - ◆ Protocol restrictions (28%)
 - ◆ Insurance plan restrictions (27%)
 - ◆ ANC level requirements (24%)
 - ◆ Formulary restrictions (23%)
 - ◆ Prior authorization requirements (20%).
- 19 out of 20 patients had insurance that covers the cost of CSF treatment.
- The insurance coverage for 9 out of 20 patients included Medicare and/or Medicaid.
- For more than 17 out of 20 patients (88%), CSFs were administered to prevent or actively treat chemotherapy-induced neutropenia (CIN). Other frequently occurring medical conditions were:
 - ◆ Myelodysplastic syndrome (4%)
 - ◆ Hematological mobilization (3%)
 - ◆ Hematological transplant (2%)
 - ◆ AML supportive care (non-CIN) (.6%)
 - ◆ HIV/AIDS-induced neutropenia (.6%)
 - ◆ Immunotherapy for prostate cancer (.6%).

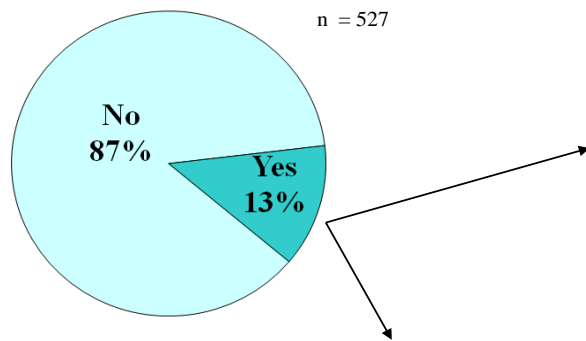
- The most frequent types of tumor or cancer treated among cancer patients were:
 - ♦ Breast cancer (31% of cancer patients)
 - ♦ Lung cancer (16%)
 - ♦ Non-Hodgkin's lymphoma (NHL) (12%)
 - ♦ Hodgkin's lymphoma (6%)
 - ♦ Lymphoma (other) (6%)
 - ♦ Prostate cancer (5%).
- About 2 of 5 CSF cancer patients (41%) were in stage IV during CSF treatment compared with 31%

in stage III, 24% in stage II, and 4% in stage I.

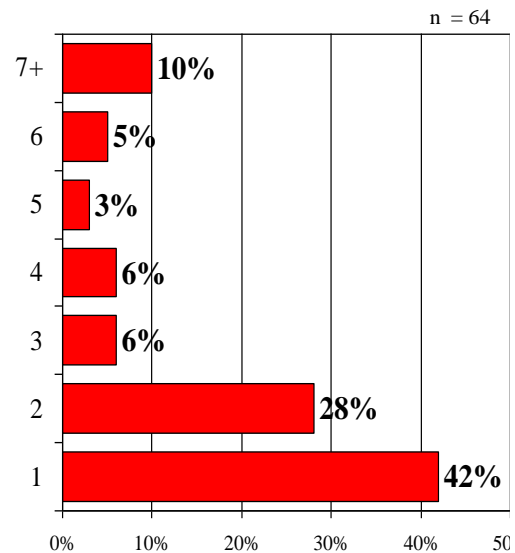
- Only 13% of patients experienced infection. When an infection occurred, it was relatively unlikely to have been after administration of a CSF (16%). Nearly 5 of 7 patients (70%) with an infection experienced it in cycles 1 or 2 of chemotherapy (see figure below).
- Physician expectations for CSF usage were met in more than 97% of CSF administrations (1,478 out of 1,521 administrations).

Infections and Temporal Relationship to Chemotherapy and CSF

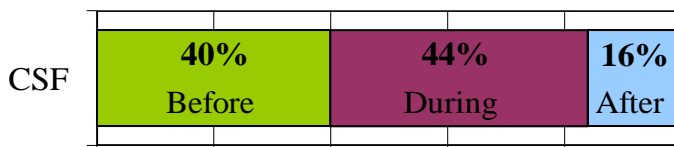
Did patient experience infection?



Chemotherapy Cycle When Infection Occurred



CSF Timing When Infection Occurred



About the Authors

Thomas Orsagh, Ph.D., is an internationally recognized economist who has made numerous scientific contributions during his career. He attended the Wharton School and has a Ph.D. from the University of Pennsylvania. He has served on the faculties of the University of Pennsylvania, Lehigh University, the University of Karlsruhe in Germany, and the University of North Carolina, Chapel Hill. He was a Fulbright Research Scholar, was an editor of the **Southern Economics Journal**, and served on a national Presidential Task Force.

Jack R. Gallagher, Ed.D., is a behavioral modeling scientist with more than 25 years' experience in medical and systems research. He was on the University of Virginia School of

Medicine faculty and directed a five-university research consortium. He has published scientific papers, presented at numerous national and international conferences, and has served on the editorial review boards of two national journals. He wrote the book **Changing Behavior: How and Why**.

Kylee Heap has managed a wide range of medical research studies, including studies on basal cell carcinoma, malignant melanoma, renal cell carcinoma, leukemia, NHL, pediatric cancers (leukemia and solid/liquid tumors), prostate cancer, hematopoietic transplantations and PBS mobilizations. She has also managed studies of likely market performance of various investigational drugs and medical devices.