



64th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY

903.HEALTH SERVICES AND QUALITY-MYELOID MALIGNANCIES

Healthcare Utilization Among Patients with Advanced Systemic Light Chain Amyloidosis

Morie A. Gertz, MD MACP¹, Rafat Abonour, MD², Sarah N. Gibbs, MPH^{3,*}, Muriel Finkel^{4,*}, Heather Landau, MD⁵, Suzanne Lentzsch, MD PhD⁶, Grace Lin, MD MBA^{1,*}, Anuj Mahindra, MD⁷, Tiffany P. Quock, PhD^{8,*}, Cara A. Rosenbaum, MD^{9,*}, Michael Rosenzweig, MD MS¹⁰, Surbhi Sidana, MD¹¹, Sascha A. Tuchman, MD MHS¹², Ronald Witteles, MD^{11,*}, Irina Yermilov, MD MPH, MS^{3,*}, Michael S. Broder, MD MSHS^{3,*}

¹Mayo Clinic, Rochester, MN

²Indiana University, Indianapolis, IN

³PHAR (Partnership for Health Analytic Research), Beverly Hills, CA

⁴Amyloidosis Support Groups Inc., Wood Dale, IL

⁵Memorial Sloan Kettering Cancer Center, New York, NY

⁶Columbia University, New York, NY

⁷Scripps Clinic, La Jolla, CA

⁸Prothena, South San Francisco, CA

⁹Weill Cornell Medicine, New York, NY

¹⁰City of Hope, Duarte, CA

¹¹Stanford University, Palo Alto, CA

¹²Division of Hematology, The University of North Carolina at Chapel Hill, Chapel Hill, NC

*Asterisk with author names denotes non-ASH members.

Abstract Introduction

Information on health service use in advanced systemic light chain (AL) amyloidosis, a rare hematologic disease, is outdated and non-specific, in part because there was no ICD-10 code for AL amyloidosis when prior studies were conducted. While there is now an ICD-10 code for AL amyloidosis, healthcare claims datasets do not include lab results and therefore lack Mayo stage (disease severity). Given the limitations of prior real-world studies, we used a validated consensus method to estimate healthcare utilization in patients with advanced AL amyloidosis during different phases of treatment.

Methods

We used a RAND/UCLA modified Delphi panel method to estimate healthcare utilization in patients with AL amyloidosis including those with advanced stage disease. We defined advanced patients in two ways: Mayo stage 3 or 4 during first-line therapy or those with signs of cardiac involvement (i.e., elevated troponin and/or NT-proBNP) after completing first-line therapy. We conducted the following steps: 1) expert panelists (9 hematologists, 2 cardiologists, 1 patient advocate) with a median 15 years' experience treating and/or managing AL amyloidosis were provided a systematic literature review on utilization in AL amyloidosis; 2) panelists independently estimated utilization (inpatient, outpatient, testing, chemotherapy, procedures) during two treatment phases (the 1-year period after starting first-line therapy and the 1-year period after completing first-line therapy) among advanced patients with varied hematologic treatment response (complete or very good partial response [CR/VGPR], partial or no response); 3) we discussed areas of disagreement during a moderated meeting; 4) panelists completed ratings independently a second time following this discussion.

Results

During the 1-year period after starting first-line therapy, 1-5% of patients with advanced AL amyloidosis were expected to be treated with a hematopoietic stem cell transplantation (HSCT). Half (45-55%) were expected to have ≥ 1 hospital admission and 28-38% were expected to have ≥ 2 hospital admissions, depending on the presence of non-cardiac symptomatic organ involvement. Patients were also expected to have high rates of other utilization in the year after starting first-line therapy: ~25% would receive a bone marrow biopsy, 12-15% would receive a cardiac pacemaker placed, and up to 7% would begin chronic dialysis. In the year after completing first-line therapy, 10% with a CR/VGPR and 15-20% with partial or no response

were expected to have ≥ 2 hospital admissions. High rates of other utilization were expected to continue, especially for advanced patients with partial or no response (50% would receive a bone marrow biopsy and up to 10% would receive a cardiac pacemaker or begin chronic dialysis).

Conclusions A diverse panel of experts, guided by a review of the literature and clinical experience, used the modified Delphi panel method to estimate healthcare utilization in patients with advanced AL amyloidosis at different points in the disease course. During the year after starting first-line therapy, advanced patients were expected to have high rates of hospitalization (traditionally the most expensive type of healthcare utilization), were not considered to be good candidates for HSCTs and had high rates of other utilization. Utilization remained high in advanced patients after completing first-line therapy especially for those with partial or no response. The development of new treatment options that can induce CR/VGPR and facilitate organ recovery or improve function may lead to decreased utilization.

Disclosures **Gertz:** *Johnson & Johnson:* Other: personal fees; *Abbvie:* Membership on an entity's Board of Directors or advisory committees, Other: personal fees from Data Safety Monitoring Board; *Juno:* Other: personal fees; *Physicians Education Resource:* Other: personal fees; *Ashfield:* Other: personal fees; *Aptitude Healthgrants:* Other: personal fees; *Janssen:* Other: personal fees; *Sanofi:* Other: personal fees; *Prothena:* Other: personal fees; *Ionis/Akcea:* Other: personal fees; *Celgene:* Other: personal fees; *Research to Practice:* Other: personal fees; *Sorrento:* Other: personal fees; *i3Health:* Other: Development of educational materials for i3Health. **Abonour:** *Bristol Myers Squibb:* Honoraria, Research Funding; *Janssen:* Honoraria, Research Funding, Speakers Bureau; *Amgen:* Honoraria; *Takeda:* Honoraria, Research Funding; *GSK:* Honoraria, Research Funding; *Prothena:* Honoraria. **Gibbs:** *Prothena:* Other: I am an employee of PHAR, LLC which was paid by Prothena to conduct the research described in the abstract; *Amgen:* Other: I am an employee of PHAR, LLC which was paid by Amgen to conduct the research related to the work described in the abstract; *BioMarin Pharmaceuticals:* Other: I am an employee of PHAR, LLC which was paid by BioMarin Pharmaceuticals to conduct the research related to the work described in the abstract; *Bristol Myers Squibb:* Other: I am an employee of PHAR, LLC which was paid by Bristol Myers Squibb to conduct the research related to the work described in the abstract; *Celgene:* Other: I am an employee of PHAR, LLC which was paid by Celgene to conduct the research related to the work described in the abstract; *Delfi Diagnostics:* Other: I am an employee of PHAR, LLC which was paid by Delfi Diagnostics to conduct the research related to the work described in the abstract; *Dompe:* Other: I am an employee of PHAR, LLC which was paid by Dompe to conduct the research related to the work described in the abstract; *Eisai:* Other: I am an employee of PHAR, LLC which was paid by Eisai to conduct the research related to the work described in the abstract; *Exact Sciences Corporation:* Other: I am an employee of PHAR, LLC which was paid by Exact Sciences Corporation to conduct the research related to the work described in the abstract; *Genentech:* Other: I am an employee of PHAR, LLC which was paid by Genentech to conduct the research related to the work described in the abstract; *Gilead:* Other: I am an employee of PHAR, LLC which was paid by Gilead to conduct the research related to the work described in the abstract; *GRAIL:* Other: I am an employee of PHAR, LLC which was paid by GRAIL to conduct the research related to the work described in the abstract; *Jazz:* Other: I am an employee of PHAR, LLC which was paid by Jazz to conduct the research related to the work described in the abstract; *Kite:* Other: I am an employee of PHAR, LLC which was paid by Kite to conduct the research related to the work described in the abstract; *Nobelpharma:* Other: I am an employee of PHAR, LLC which was paid by Nobelpharma to conduct the research related to the work described in the abstract; *Novartis:* Other: I am an employee of PHAR, LLC which was paid by Novartis to conduct the research related to the work described in the abstract; *Otsuka:* Other: I am an employee of PHAR, LLC which was paid by Otsuka to conduct the research related to the work described in the abstract; *Recordati:* Other: I am an employee of PHAR, LLC which was paid by Recordati to conduct the research related to the work described in the abstract; *Regeneron:* Other: I am an employee of PHAR, LLC which was paid by Regeneron to conduct the research related to the work described in the abstract; *Sanofi US Services:* Other: I am an employee of PHAR, LLC which was paid by Sanofi US Services to conduct the research related to the work described in the abstract; *Takeda Pharmaceuticals USA:* Other: I am an employee of PHAR, LLC which was paid by Takeda Pharmaceuticals USA to conduct the research related to the work described in the abstract. **Landau:** *Karyopharm:* Consultancy; *Memorial Sloan Kettering Cancer Center:* Current Employment; *Pfizer:* Consultancy; *Celgene:* Consultancy; *Juno:* Consultancy; *Takeda Pharmaceuticals:* Consultancy, Other: grants/pending grants; *Legend Biotech USA Inc:* Consultancy; *Caelum Biosciences:* Consultancy; *Janssen:* Consultancy; *Alexion:* Other: grants/pending grants; *Janssen Scientific Affairs, LLC:* Other: grants/pending grants; *Prothena:* Honoraria. **Lentzsch:** *Zentalis:* Research Funding; *Oncopeptides:* Consultancy; *Sanofi:* Consultancy, Research Funding; *GlaxoSmithKline:* Consultancy; *Janssen:* Consultancy; *Nataea:* Consultancy; *Takeda:* Consultancy; *Pfizer:* Consultancy; *Poseida:* Other: Equity ownership; *Magenta:* Other: Equity Ownership; *Caelum Bioscience:* Consultancy, Other: Stock ownership; *Peerview:* Speakers Bureau; *Clinical Care Options:* Speakers Bureau; *Prothena:* Honoraria. **Lin:** *Prothena:* Honoraria. **Mahindra:** *Takeda:* Consultancy, Honoraria; *ADC Therapeutics:* Consultancy, Honoraria; *Gilead:* Consultancy, Honoraria; *Oncopeptides:* Consultancy, Honoraria; *Janssen:* Honoraria, Speakers Bureau; *Sanofi:* Honoraria, Speakers Bureau; *Prothena:* Honoraria. **Quock:** *Prothena:* Current Employment, Current equity holder in publicly-traded company. **Rosenzweig:** *Pfizer:* Research Funding; *Akcea:* Speakers Bureau; *Prothena:* Honoraria; *Janssen, BMS, Akcea:* Research Funding, Speakers Bureau; *Janssen:* Research Funding, Speakers Bureau; *Bristol Myers Squibb:* Speakers Bureau. **Sidana:** *Allogene:* Research Funding; *Janssen:* Consultancy, Research Funding; *Sanofi:* Consultancy; *Oncopeptides:* Consultancy; *Bristol Myers Squibb:* Consultancy, Research Funding; *Magenta Therapeutics:* Consultancy, Research Funding; *Prothena:* Honoraria. **Tuchman:** *Prothena:* Honoraria; *Shattuck Labs:* Honoraria; *Janssen:* Honoraria. **Witteles:** *Prothena:* Honoraria; *Pfizer:* Honoraria; *Alnylam:* Honoraria; *BridgeBio:* Honoraria; *Janssen:* Honoraria; *Ionis:* Honoraria; *Intelia:*

Honoraria; *Novo Nordisk*: Honoraria. **Yermilov**: *Recordati*: Other: I am an employee of PHAR, LLC which was paid by Recordati to conduct the research related to the work described in the abstract; *Regeneron*: Other: I am an employee of PHAR, LLC which was paid by Regeneron to conduct the research related to the work described in the abstract; *Otsuka*: Other: I am an employee of PHAR, LLC which was paid by Otsuka to conduct the research related to the work described in the abstract; *Novartis*: Other: I am an employee of PHAR, LLC which was paid by Novartis to conduct the research related to the work described in the abstract; *Kite*: Other: I am an employee of PHAR, LLC which was paid by Kite to conduct the research related to the work described in the abstract; *Nobelpharma*: Other: I am an employee of PHAR, LLC which was paid by Nobelpharma to conduct the research related to the work described in the abstract; *Jazz*: Other: I am an employee of PHAR, LLC which was paid by Jazz to conduct the research related to the work described in the abstract; *BioMarin Pharmaceuticals*: Other: I am an employee of PHAR, LLC which was paid by BioMarin Pharmaceuticals to conduct the research related to the work described in the abstract; *Bristol Myers Squibb*: Other: I am an employee of PHAR, LLC which was paid by Bristol Myers Squibb to conduct the research related to the work described in the abstract; *Celgene*: Other: I am an employee of PHAR, LLC which was paid by Celgene to conduct the research related to the work described in the abstract; *Delfi Diagnostics*: Other: I am an employee of PHAR, LLC which was paid by Delfi Diagnostics to conduct the research related to the work described in the abstract; *Dompe*: Other: I am an employee of PHAR, LLC which was paid by Dompe to conduct the research related to the work described in the abstract; *Eisai*: Other: I am an employee of PHAR, LLC which was paid by Eisai to conduct the research related to the work described in the abstract; *Exact Sciences Corporation*: Other: I am an employee of PHAR, LLC which was paid by Exact Sciences Corporation to conduct the research related to the work described in the abstract; *Genentech*: Other: I am an employee of PHAR, LLC which was paid by Genentech to conduct the research related to the work described in the abstract; *Gilead*: Other: I am an employee of PHAR, LLC which was paid by Gilead to conduct the research related to the work described in the abstract; *GRAIL*: Other: I am an employee of PHAR, LLC which was paid by GRAIL to conduct the research related to the work described in the abstract; *Amgen*: Other: I am an employee of PHAR, LLC which was paid by Amgen to conduct the research related to the work described in the abstract; *Prothena*: Other: I am an employee of PHAR, LLC which was paid by Prothena to conduct the research described in the abstract; *Sanofi US Services*: Other: I am an employee of PHAR, LLC which was paid by Sanofi US Services to conduct the research related to the work described in the abstract; *Takeda Pharmaceuticals USA*: Other: I am an employee of PHAR, LLC which was paid by Takeda Pharmaceuticals USA to conduct the research related to the work described in the abstract. **Broder**: *Takeda Pharmaceuticals USA*: Other: I am an employee of PHAR, LLC which was paid by Takeda Pharmaceuticals USA to conduct the research related to the work described in the abstract; *Jazz*: Other: I am an employee of PHAR, LLC which was paid by Jazz to conduct the research related to the work described in the abstract; *Kite*: Other: I am an employee of PHAR, LLC which was paid by Kite to conduct the research related to the work described in the abstract; *Nobelpharma*: Other: I am an employee of PHAR, LLC which was paid by Nobelpharma to conduct the research related to the work described in the abstract; *Novartis*: Other: I am an employee of PHAR, LLC which was paid by Novartis to conduct the research related to the work described in the abstract; *Otsuka*: Other: I am an employee of PHAR, LLC which was paid by Otsuka to conduct the research related to the work described in the abstract; *Recordati*: Other: I am an employee of PHAR, LLC which was paid by Recordati to conduct the research related to the work described in the abstract; *Regeneron*: Other: I am an employee of PHAR, LLC which was paid by Regeneron to conduct the research related to the work described in the abstract; *Sanofi US Services*: Other: I am an employee of PHAR, LLC which was paid by Sanofi US Services to conduct the research related to the work described in the abstract; *Delfi Diagnostics*: Other: I am an employee of PHAR, LLC which was paid by Delfi Diagnostics to conduct the research related to the work described in the abstract; *Celgene*: Other: I am an employee of PHAR, LLC which was paid by Celgene to conduct the research related to the work described in the abstract; *Bristol Myers Squibb*: Other: I am an employee of PHAR, LLC which was paid by Bristol Myers Squibb to conduct the research related to the work described in the abstract; *BioMarin Pharmaceuticals*: Other: I am an employee of PHAR, LLC which was paid by BioMarin Pharmaceuticals to conduct the research related to the work described in the abstract; *Amgen*: Other: I am an employee of PHAR, LLC which was paid by Amgen to conduct the research related to the work described in the abstract; *Prothena*: Other: I am an employee of PHAR, LLC which was paid by Prothena to conduct the research described in the abstract; *GRAIL*: Other: I am an employee of PHAR, LLC which was paid by GRAIL to conduct the research related to the work described in the abstract; *Gilead*: Other: I am an employee of PHAR, LLC which was paid by Gilead to conduct the research related to the work described in the abstract; *Genentech*: Other: I am an employee of PHAR, LLC which was paid by Genentech to conduct the research related to the work described in the abstract; *Exact Sciences Corporation*: Other: I am an employee of PHAR, LLC which was paid by Exact Sciences Corporation to conduct the research related to the work described in the abstract; *Eisai*: Other: I am an employee of PHAR, LLC which was paid by Eisai to conduct the research related to the work described in the abstract; *Dompe*: Other: I am an employee of PHAR, LLC which was paid by Dompe to conduct the research related to the work described in the abstract.

<https://doi.org/10.1182/blood-2022-156832>