ABSTRACT: Observational Studies Evaluating Biosimilar Agents: A Focused Literature Review

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Objectives:

Cost-containment in health care paired with global regulatory approvals of biosimilar agents pave the way for less expensive alternatives to complex biologics. Regulators and payers increasingly demand robust safety and effectiveness data in real-world settings. Our objective was to identify and describe characteristics of observational studies involving biosimilars.

Methods:

Embase was searched using the keywords: biosimilar agent, observational study, registry, drug efficacy/post-marketing surveillance, and health resource utilization (HRU). Publications not referring to observational studies, duplicates and review/discussion papers were excluded. Key data abstracted included country, biosimilar/reference products, therapeutic area, study design, number of patients/sites, follow-up period, and outcomes of interest.

Results:

Of 188 abstracts reviewed, 17 met inclusion criteria. Fifteen studies (88%) were conducted in the European Union (EU) (Italy/France/Germany/Spain/other) and two (12%) outside EU. Therapeutic areas included chemotherapy-induced febrile neutropenia (n=7), chronic kidney disease/anaemia (n=4), chemotherapy-induced anaemia (n=2), and other (n=5). Filgrastim was the most frequent reference drug (n=8) with 3 different biosimilars cited. Erythropoiesis Stimulating Agents (ESA) appeared in 5 studies with 2 different biosimilars. Infliximab (n=1) and somatropin (n=1) were cited with two and one biosimilars, respectively. Follow-up time ranged from 12 weeks to 10 years. All but two studies were regulator-mandated. Distribution by study design was: twelve (70%) prospective, three (18%) retrospective, one (6%) survey, and one (6%) registry. Outcomes included safety (n=16), effectiveness (n=16), patterns of
care (n=6), HRU (n=3), physicians’ perspective (n=2), and Quality of Life (QoL) (n=1). All studies (n=11) reporting results on effectiveness/safety of a biosimilar found it in line with reference product.

**Conclusions:**

Almost all observational studies of biosimilars were mandated by regulators in the EU (where biosimilars were approved first) and assessed real-world safety and effectiveness. Study infrastructures were often leveraged by including other outcomes (HRU, QoL), allowing the generation of real-world health economic and outcome evidence.
List of Articles Reviewed