Poster #19 | Single patient compassionate use of an investigational targeted therapy for recurrent metastatic serous uterine carcinoma: A case report and quality improvement analysis

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Topic: Quality & Healthcare systems

Objectives
The Federal Drug Administration’s (FDA) Expanded Access Program serves as the primary pathway for severely ill patients, who are not candidates for clinical trials and have exhausted all treatment options, to access potentially life-prolonging investigational treatments. While much has been written about the ethics of compassionate use, there is limited guidance in the literature to help physicians navigate the application process. The aim of our project was to outline our own experience with obtaining compassionate use, and to use quality improvement tools to help identify barriers and opportunities for improvement.

Methods
We constructed a Process Map and a Venn Diagram using our own patient experience to identify the steps and barriers involved in obtaining compassionate use.

Results
We report a case of a 35-year-old woman with recurrent uterine serous carcinoma who despite failing all lines of conventional chemotherapy, had a near complete response to a targeted therapy on a clinical trial. Unfortunately, she developed persistent thrombocytopenia and was taken off trial. She then progressed rapidly, but her functional status remained excellent and patient desired to pursue compassionate use of this targeted therapy. Our Process Map demonstrates that it took 3 months from the patient’s first inquiry, to when she was able to initiate treatment. The majority of this time, about 2.5 months, was spent requesting and awaiting drug manufacturer approval. We were then able to obtain FDA and Internal Review Board approval within 1.5 weeks, and started treatment 1 week later. Our Process Map identifies 23 process steps and 18 individuals/organizations involved in obtaining compassionate use. We identify 12 barriers, which we organize into 3 major categories—Time, Communication, and Physician Resources. Using a Venn Diagram (Figure 1), we demonstrate that most of these barriers are multi-dimensional and involve the barrier of “Time”

Conclusions
Obtaining compassionate use of an investigational therapy is a multi-step process that requires collaboration between multiple individuals and organizations on a national and institutional level, while balancing the expectations and needs of a severely ill patient. We identify 3 main categories of barriers, which cluster around the barrier of “Time” We believe that by developing better resources to help physicians navigate this complex process, the barrier of “Time” could be greatly reduced. As the field of targeted therapies continues to rapidly expand, we anticipate a growing need and demand for compassionate use.

Abstract Table or Graph
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