FIGURE 17

## PHASES OF CLINICAL TRIALS

| Phase I | Phase II | Phase III | Phase IV |
|---|---|---|---|
|  |  |  |  |
| **Safety and dosage** | **Safety and efficacy** | **Therapeutic efficacy compared to standard of care** | **Postmarketing studies providing effectiveness or "real-world" data** |
|  |  |  |  |
| **Tens of patients** | **Hundreds of patients** | **Thousands of patients** | **Thousands of patients** |

Oncology clinical trials—the types of clinical studies that evaluate the efficacy and safety of potential new therapeutics for treating cancer patients—have traditionally been conducted in three successive phases, each with an increasing number of patients. Phase I studies determine the optimal dose of an investigational anticancer therapeutic, how humans metabolize it, and any potentially harmful side effects. Phase II studies determine the initial efficacy of an investigational therapy in humans while continually monitoring for potential toxicities. Phase III studies are large trials designed to determine therapeutic efficacy of the new drug in comparison to standard of care. When successful, the results of these trials can be used by the U.S. Food and Drug Administration (FDA) to approve new therapeutics or new indications for existing therapeutics. Phase IV studies are conducted after a therapy is provisionally approved by FDA and provide additional effectiveness or "real-world" data on the therapy.

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