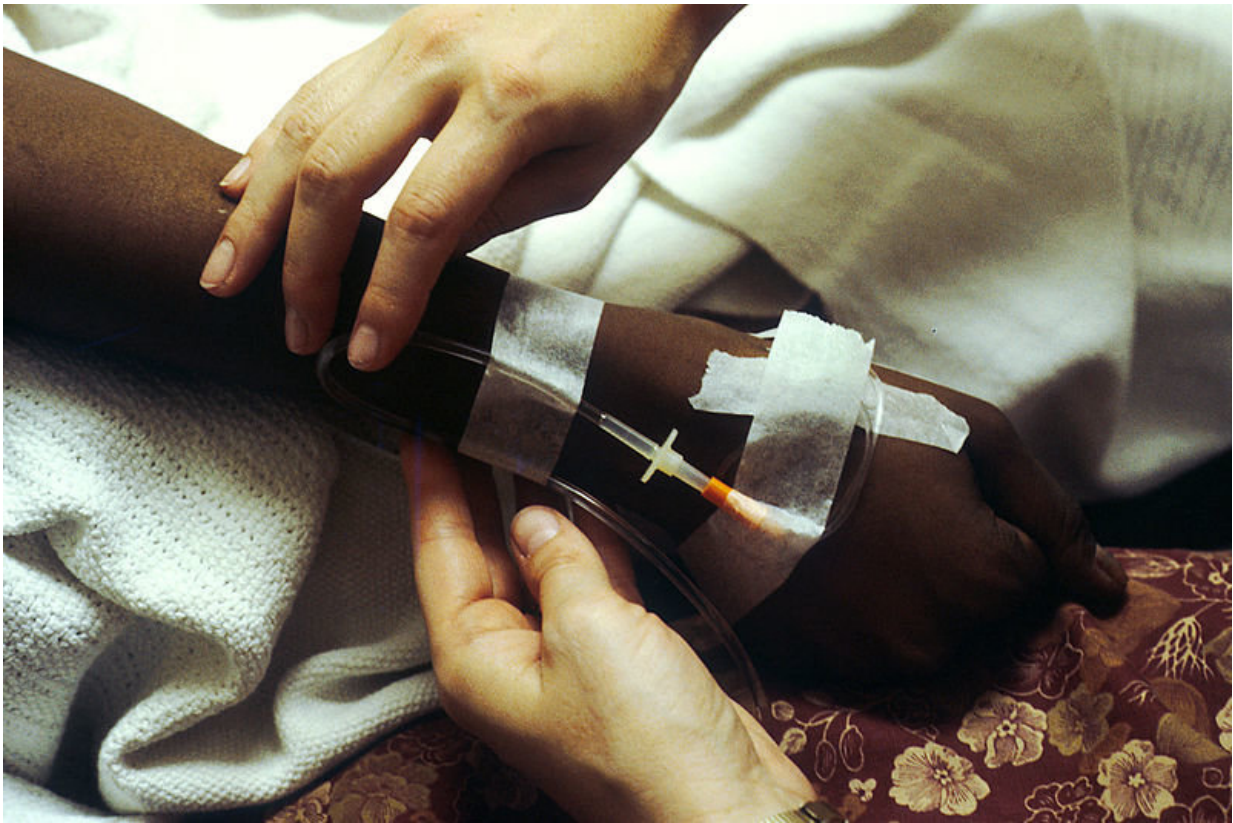


# Study weighs risks and benefits of phase I trials in pediatric cancer

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Systematic review and meta-analysis finds 1 in 10 children who enroll in pediatric phase I cancer trials are improved after the trial, and 1 in 50 die from drug-related complications. Credit: National Cancer Institute, Wikimedia

On average, 1 in 10 children who enroll in pediatric phase I cancer trials

are improved after the trial, and 1 in 50 die from drug-related complications, according to a new systematic review and meta-analysis published this week in *PLOS Medicine* by Jonathan Kimmelman from McGill University, Canada, and colleagues.

Pediatric phase I [trials](#) are critical for establishing the safety and dosing of [anti-cancer drugs](#) in children. National and international regulations, however, establish limits on allowable risk for research involving minors. In the new study, researchers systematically searched for pediatric phase I [cancer](#) studies published between 2004 and 2015. They identified 170 studies involving 4,604 patients, and pooled the objective response rates and the occurrence of grade 3, 4, or 5 (fatal) drug-related adverse events.

Overall, the response rate among all trials was 10.29% (95% CI 8.33 to 12.25) but the rate was significantly different for solid tumors (3.17, 95% CI 2.62 to 3.72) compared to hematological malignancies (27.90, 95% CI 20.53 to 35.27). The overall rate of fatal grade 5 adverse events was 2.09% (95% CI 1.45 to 2.72) and there was an average of 1.32 grade 3 and 4 drug-related adverse events per person. On the whole, adverse event and response rates were similar to those seen in adult phase I cancer trials.

The authors note several limitations to their study, including heterogeneity in the types of cancer and treatment in the included trials, reliance on published data only, and low-quality or incomplete reporting of some trial outcomes.

"Our data, coupled with careful ethical analysis, provide an empirical basis for further discussions about the therapeutic status of phase I trials in children," the authors say. "In particular, they provide evidence for refining risk/benefit in [phase](#) I trials and identifying those studies that present greater challenges for meeting standards of acceptable risk in

children."

**More information:** Waligora M, Bala MM, Koperny M, Wasylewski MT, Strzebonska K, Jaeschke RR, et al. (2018) Risk and surrogate benefit for pediatric Phase I trials in oncology: A systematic review with meta-analysis. *PLoS Med* 15(2): e1002505.  
[doi.org/10.1371/journal.pmed.1002505](https://doi.org/10.1371/journal.pmed.1002505)

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