



Roche

EGFR tyrosine kinase domain with erlotinib.

Soloway, MD, of Memorial Regional Hospital in Hollywood, FL.

McKiernan agrees. A clinical trial “is a no-brainer,” he says. ■

## EMA to Publish Clinical Trial Reports

In a move toward full transparency, European regulators will soon publish clinical trial reports on all drugs approved for marketing in the European Union (EU).

The policy, which was approved in principle on June 12 by the European Medicines Agency’s (EMA) management board, is expected to be formally adopted at the board’s October 2 meeting. The move will allow researchers to download clinical trial reports from the organization’s website, enabling them to analyze, share, and compare trial data once the decision-making process for the medicine in question has been completed.

“We began giving access to clinical study reports in 2010, and we’ve released well over 2 million pages of reports since then,” says Martin Harvey, EMA spokesperson. “The big difference now is that instead of waiting for researchers to formally request access to documents, we will proactively publish them on our website.”

For now, reports published on the website will not include anonymous patient-level data, which contain complete information on each participant in a trial (as opposed to aggregate or summary data). Researchers can still obtain such data but must formally request it until the EMA works out privacy-protection issues and finalizes guidelines in a second phase of the policy.

The EMA policy has provoked debate since it was first introduced in May 2013. Although researchers and patient

groups have pushed for full transparency—provided that the identities of patients are protected—pharmaceutical companies have objected to sharing data with competitors. However, EMA regulators have contended that open access to data ultimately benefits all sides.

“Contrary to industry fears, we argue that access to full—though appropriately de-identified—data sets from clinical trials will benefit the research-based biopharmaceutical industry,” wrote EMA executive director Guido Rasi, MD, and colleagues last year (*N Engl J Med* 2013;369:1577–9). “We predict that it will help to increase the efficiency of drug development, improve cost-effectiveness, improve comparative-effectiveness analysis, and reduce duplication of effort among trial sponsors.”

To address industry concerns, the policy allows companies to request redaction of information deemed commercially confidential, such as details of manufacturing processes or exploratory endpoints that a company may have been pursuing at the time of submission but have not yet been approved, says Harvey. The final rule will contain full details on what types of requests the EMA will consider.

Although some critics of the policy worry that companies will have too much control over withholding data, Harvey notes that most of the data in clinical study reports does not qualify as commercially confidential.

“Our redaction principles will lay out the only areas where we are prepared to consider exceptions, and the final decision is ours,” he says. In addition, the extent and type of information redacted will be visible on the EMA website.

The EMA is the first major drug regulatory agency to propose publishing participant-level clinical trial reports on specific approved drugs. The FDA has proposed making trial data available without linking it to specific products or applications by, for example, releasing certain data from a random sample or pooling data within one product class.

“The FDA’s concept is to develop a framework to make publicly available non-summary safety and effectiveness data that has been de-identified and masked,” says Stephen King, spokesperson for the FDA’s Center for Drug Evaluation and Research. “The data

made available under this framework could be used, for example, to help further understanding of potential biomarkers, endpoints, and drug-development tools to improve public health and stimulate innovation in medical product development.” ■

## CTCs Could Guide Cancer Therapy

Researchers have shown that rare tumor cells circulating in a patient’s blood can be cultured and genetically analyzed. The approach, described in *Science* in July, demonstrates that it may be possible to use a simple blood draw to track a cancer as it evolves and adjust treatment accordingly (*Science* 2014;345:216–20).

Scientists isolated circulating tumor cells (CTC)—cancer cells shed by solid tumors—from the blood of 36 patients with metastatic estrogen receptor-positive breast cancer. CTC cell lines from six patients were successfully grown in the lab and screened for mutations in 1,000 cancer-associated genes, revealing new mutations that were not present in the primary tumors.

Tumors have learned to adapt to effective treatments by changing their genetic make-up, says co-senior author Daniel Haber, MD, PhD, director of the Massachusetts General Hospital (MGH) Cancer Center in Boston. Indeed, CTCs from three patients treated extensively with estrogen-blocking aromatase inhibitors tested positive for an uncommon estrogen receptor mutation. Another patient’s cancer developed new mutations in both *PI3K* and *FGFR*.

Subsequent testing found various targeted drugs could inhibit cancer growth in patient-derived cell lines and in mice with tumors developed from each patient’s CTCs.

The existence of CTCs has been known for nearly 150 years. They are exceedingly rare, with a single cancer cell drifting among a billion healthy blood cells. To date, they have been useful only in predicting a patient’s prognosis.

In the study, CTCs were captured using the MGH-developed CTC-iChip, which Johnson & Johnson (J&J) will develop for commercial use. The device can capture CTCs in nearly 80% of patients with metastatic cancer, whereas