Editorial

Stand up for the FDA

Of the many upheavals in the US government in the first three months of the Trump presidency, the one that is likely to affect the biopharma industry most directly is the gutting of the staff at the Food and Drug Administration, the world's premier drug regulator. The agency has a long history of protecting public health illustrated by the intervention of reviewer Frances Oldham Kelsey in the 1960s to prevent thalidomide, a treatment for nausea during pregnancy, from being approved for the US market.

The drug was already in use in Europe, but its toxic effects had not been immediately recognised. The developer wanted access to the US market, but Dr Kelsey refused on the grounds that the scientific evidence for approval was inadequate. In the fullness of time, it became clear that thalidomide was the cause of limb, eye, urinary tract, and heart problems that afflicted thousands.

What qualities of mind and spirit led Dr Kelsey to stand up to pressure from doctors who wanted the medicine and to the companies that produced it? We will probably never know. But there certainly was a public health mission that she understood and which was embedded in the culture of the FDA that made it possible for her to act with conscience.

This structure is now under threat as the new administration takes steps to brutally downsize the agency and remove its top management with unprecedented haste. In late March, the Department of Health and Human Services (HHS), the FDA's governing body, announced plans to reduce the department's workforce by 20,000 including the elimination of 3,500 jobs at the FDA. Effective 7 April, the FDA had lost 28 senior leaders, one of whom, Peter Marks, was director of the Center for Biologics Evaluation and Research, one of the most innovative divisions of the FDA.

Dr Marks was in charge of vaccines and cell and gene therapies. His departure was reportedly triggered by a disagreement with the HHS secretary Robert F Kennedy Jr over vaccines. But in his resignation letter Dr Marks also spoke about an "unprecedented assault on scientific truth that has adversely impacted public health in our nation."

It is hoped that Martin Makary, the new FDA commissioner, will be alert to this threat and take steps to protect the FDA's independence and integrity. But he should not be alone. The industry has a role to play as well. Some 70% of the CBER budget is funded by user fees. This represents leverage, if the industry chooses to use it, to stand up for good governance and respect the work of the professionals at the FDA.

– By Victoria English, 24 April 2025

Contents