Threat posed by unproven drugs in medical oncology

Noam Pondé,1 Felipe Ades,2 Evandro de Azambuja1

Despite the advances obtained with new agents developed in recent years in oncology, most patients with metastatic solid tumours remain incurable. Oncologists face these patients on a daily basis and know that often this situation leads to denial and the search for non-scientific, sometimes called ‘alternative’, treatments.1 Although they lack scientific evidence they hold the advantage of being able to ‘promise’ results to patients. Although patients have the right to seek such treatments, at a societal level and, more significantly, at the level of healthcare system planning, the widespread use of such agents can pose a threat when they receive approval by regulatory agencies, such as in the case of the agent Ukrain, or when political pressure and legal actions are brought to bear to bypass regulatory systems, such as in the case of phosphoethanolamine (PHOS).

Ukrain, developed in Austria by Nowicky Pharma, was approved by regulatory agencies in countries such as Ukraine and UAE even when available data from preclinical studies and small clinical trials did not confirm its efficacy.2 The journey of a drug candidate from bench to clinic is long and fraught with perils, leading most compounds that show activity in vitro to be abandoned for lack of in vivo efficacy or for excessive toxicity.3 Therefore, regulatory agencies such as the Food and Drug Administration (FDA) or the European Medicine Association (EMA) demand the strictest adherence to accepted scientific standards (from in vitro essays to phase 3 randomised trials) and to Good Clinical Practices (GCP), before ever considering registering a new agent. When regulatory agencies lower their standards, patients might be exposed to treatments with unknown benefit or toxicity.

Meanwhile, in Brazil, PHOS was being distributed to patients as a cure for nearly all types of cancers based on preclinical findings.4 Once supply was interrupted by the authorities, patients went to the courts to continue treatment and generated such a loud public outcry that, in a scant few months, both houses of Congress approved a law that legalised the distribution of PHOS without authorisation from regulatory agencies as long as a physician prescribes it, despite the opposition of the Brazilian Medical Association, of the Brazilian Society of Clinical Oncology and the regulatory agency ANVISA.5 Regulatory agencies exist, ultimately, to protect patients and society from the danger posed by exposure to ineffective or toxic substances by having the legal power to classify a candidate drug as a scientific validated therapy option, or not, therefore acting as gatekeeper for the healthcare system. When this essential legal power is blatantly disregarded, as in case of PHOS, the healthcare system is placed in jeopardy, since legal courts and political pressure may then become tools to authorise any kind of treatment, making planning and cost controlling impossible.

Both cases highlight the importance of scientific research and education, at the level of the general population, physicians, legal and political representatives. They also bring to the fore the central role strong and independent regulatory agencies play in the process of guaranteeing the safety and efficacy of drugs used by patients with cancer.

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