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Define and regulate

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- Teva Pharmaceuticals
- Nanotechnology Characterization Laboratory (NCL)
- Vifor Pharma Ltd.
- Basel University
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NEW YORK—Nanomedicine, the application of nanotechnology for the treatment, diagnosis, monitoring and control of biological systems, holds great promise for addressing some of the most challenging problems in nearly every medical specialty, and this relatively new and emerging field has the potential to generate many new opportunities for improving human health.

However, there are also concerns that the fundamentally different properties of nanoparticles compared with bulk materials may pose significant safety issues, and therefore require additional regulatory scrutiny. As part of that discussion, the New York Academy of Sciences presented a meeting, sponsored by Teva Pharmaceuticals, on nanomedicines on Nov. 21.

In part, the conference focused on current U.S. and international regulatory frameworks for nanomedicines and the needs ahead, additional safety and toxicity research needed to determine unknown properties of nanomaterials and lessons learned from featured drug discovery and development case studies of nanomedicines.

Several speakers noted that regulations need to differentiate between medicines, where risk has to be weighed against benefit, and materials, an area in which there can be a broader approach. Nanomedicines, which can combine the two areas of medicine and materials, need special regulations.

Dr. Scott McNeil, director of the Nanotechnology Characterization Laboratory (NCL), where he coordinates preclinical characterization of nanotech cancer therapeutics and diagnostics, addressed the challenges associated with characterization of nanomaterials. He explained that NCL provides independent verification of results, giving submitters “a preview of what FDA may be concerned with, based on past experience.”

NCL focuses on questions related to what McNeil calls “translatability.” That is, issues of publication vs. commercialization, manufacturing complexity, economics (such as costs to produce and potential for return on investment), quality and regulatory requirements and advantages over existing therapies. NCL scientists are responsible for testing candidate nanotech drugs and diagnostics, evaluating safety and efficacy and assisting with product development from discovery level, through scale-up and into clinical trials, and the U.S.-government funded organization has assisted in characterization and evaluation of more than 300 nanotechnology products, several of which are now in human clinical trials.

“Physicochemical characterization matters, because physical and chemical properties contribute to a nanomaterial’s biocompatibility,” McNeil explained. “You have to know what you have and the manufacturer’s specifications may not always be right. NCL performs characterization under relevant conditions. Nanomaterial safety testing is interdisciplinary, because it takes a combination of physicochemical, *in-vitro* and *in-vivo* testing to understand the results.”

In discussing nanosimilars and follow-on nano-sized therapeutics, Dr. Stefan Mühlebach of Vifor Pharma Ltd. and Basel University in Switzerland, also talked about how the NCL looks at trends across nanoparticle platforms, sees parameters that are critical to nanoparticle biocompatibility and develops assays for preclinical characterization of nanoparticles. He explained that the NCL has developed more than 40 protocols that rigorously characterize nanoparticle physicochemical properties, as well as *in-vitro* immunological and cytotoxic characteristics and ADME/Tox profiles in nonhuman animal models. These assays have undergone extensive in-house validation and are subjected to regular revision to ensure applicability to a variety of nanomaterials.

According to Mühlebach, “A new class of non-biological complex drugs (NBCDs), which also includes nanomedicines, has emerged. NBCDs are medicinal products, not being a biological medicine, where the active substance consists of different structures that can’t be isolated and fully quantitated, characterized and/or described by physicochemical analytical means.”

Therapeutic equivalence of NBCD follow-on products, including nanosimilars, cannot be assessed using standard generic or biosimilar approaches, Mühlebach explained, adding, “Generic drugs with low molecular weight have to show pharmaceutical identity and bioequivalence.”

For his part, Dr. Raj Bawa, patent agent at Bawa Biotech LLC, adjunct professor at Rensselaer Polytechnic Institute and scientific advisor to Teva Pharmaceuticals, addressed the definition of “nano.” He said that there is no universally accepted nomenclature for nanotechnology, nanoscience, nanoscale, nanomaterial, nanomedicine and nanobiotechnology, which “poses problems for regulatory bodies like the FDA.”

He added, “This is a sure recipe for conflicts and a dispute over what was meant and intended in patent claims. Precise definitions of the terms related to nanotechnology are essential to outline the regulations, formulate the legal terminology for patents and for nano commercialization. There are barriers in communications between scientists and complicated litigation when construing claims where terminology is not uniform.”

According to Bawa, “There is confusion, hype and misinformation. Is venture investment impacted and public enthusiasm stymied?” He believes that the correct approach should be a practical definition of nanotechnology, unconstrained by an arbitrary size limitation. The upper limit should be 1000 nm, especially for regulatory and IP purposes, he said.

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