

While United States is now the biggest market for medical-grade cannabis, Israel is pushing hard to position itself as a global player in this rapidly growing area of biopharmaceutical research. It is reported that about 120 research programs, including clinical trials looking at the effects of cannabis on autism, epilepsy, psoriasis, and tinnitus, are active in Israel.

The FDA has not approved marijuana as a safe and effective drug for any indication. The agency has, however, approved one specific drug product that contains the purified substance cannabidiol, one of more than 80 active chemicals in marijuana, for the treatment of seizures associated with Lennox-Gastaut syndrome or Dravet syndrome in patients 2 years of age and older. The FDA has also approved two drugs containing a synthetic version of a substance that is present in the marijuana plant and one other drug containing a synthetic substance that acts similarly to compounds from marijuana but is not present in marijuana. The FDA is aware that there is considerable interest in the use of marijuana to attempt to treat a number of medical conditions, including, for example, glaucoma, AIDS wasting syndrome, neuropathic pain, cancer, multiple sclerosis, chemotherapy-induced nausea, and certain seizure disorders.

1.1.6 Target-Based Discovery

A vital part of any small-molecule drug discovery program is hit exploration—the identification of those starting point molecules that would embark on a journey toward successful medications (however, they rarely survive this journey)—via numerous optimization, validation, and testing stages. The key element of hit exploration is the access to an expanded and chemically diverse space of drug such as molecules to choose candidates from, especially for probing novel target biology. Given that the existing compound collections at the hands of pharma were built in part based on the small-molecule designs targeting known biological targets, new biological targets require new designs and new ideas instead of recycling.

Target-based drug discovery has enabled a great expansion of chemotypes and pharmacophores available for the medicinal chemist during the past three decades. New techniques such as HTS, fragment-based screening (FBS), crystallography in combination with molecular modeling, and combinatorial and parallel chemistry have created a considerable diversity of chemical lead structures well beyond the known natural products and ligands used as chemical starting points for drug discovery in the past. Moreover, this wealth of chemotypes can now be used as a source for tool compounds to study unexplored biological space and find new drug targets or for phenotypic screening by using systems-based approaches to identify drug candidates in a target-agnostic manner.

1.1.7 High-Through Screening

Typically, the libraries are composed of the compounds synthesized over time by individual companies and influenced by a company's history; for example, Novartis has a large number of ergot compounds in its library, and Roche would have many benzodiazepines. However, as many companies work on similar targets or scaffolds, there must also be some overlap between the libraries. These libraries are a key component of the success of pharmaceutical companies; however, they have once been in