



FIGURE 1.1 Comparison of phenotype screen and drug development using drug repurposing screen, with traditional process of drug discovery. (From <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4531371/>.)

predesigned RNA sequence—“guiding” RNA (gRNA), within a longer RNA scaffold. The role of the latter is to direct Cas9 “scissors” to the exact location in the DNA of interest and modify it as desired. The CRISPR/Cas9 gene editing approach has transformed the biomedical research field forever. Compared with its “predecessors,” such as zinc finger nucleases (ZFNs) and transcription activator-like effector nucleases (TALENs), the CRISPR-Cas9 system is simpler, more precise, and relatively cheaper, making it an ideal vehicle for “playing with genome.” On October 10, 2018, the FDA announced that it has lifted the clinical hold and accepted the Investigational New Drug (IND) application for CTX001 for the treatment of sickle cell disease (SCD), the first CRISPR product.

With a number of existing strategies to influence the immune systems, such as using checkpoint inhibitors, vaccines, and monoclonal antibodies, it is the chimeric antigen receptor (CAR) T-cell therapy where a patient’s own immune cells (or cells from another donor) are removed and modified, so that they can recognize and attack the patient’s particular cancer. The engineered CAR T-cells are then grown in the lab to reach billions in numbers, followed by infusion back into the patient’s body. The CAR T-cell technology has already proved to be extremely promising in the case of hard-to-treat lymphoma and is considered an ultimate future of the immunotherapy field. The FDA has approved two CART products.

1.1.2 Microbiome

The microbiome trend is growing rapidly and can possibly become one of the major game-changers in the biopharmaceutical industry. Microbiota is the ecosystem of more than 100 trillion microorganisms living inside our body or on the skin, coexisting