



**Figure 10.** Potential applications of iPSC technology. Shown are the potential applications of iPSC technology for cell therapy and disease modeling using SMA as an example. In SMA patients, motor neurons are afflicted and die causing the devastating symptoms of the disease. SMA-specific iPSCs could be coaxed into motor neurons in vitro to establish a culture model of the disease that may lead to the identification of novel drugs that prevent the abnormal death of motor neurons in patients. Alternatively, the disease-causing mutation could be repaired, if known (in this case, *SMN* gene), in iPSCs by gene targeting before their differentiation into healthy motor neurons and transplantation into the patient's brain. (Adapted, with permission, from Stadtfeld and Hochedlinger 2010.)