Record Nr. UNINA9910792058803321 Autore Cohen-Haguenauer Odile Titolo The clinibook [[electronic resource]]: clinical gene transfer: state of the art / / edited by Odile Cohen-Haguenauer Paris, : EDP Sciences, 2012 Pubbl/distr/stampa **ISBN** 1-299-27682-2 2-84254-237-1 Descrizione fisica 1 online resource (589 p.) Altri autori (Persone) Cohen-HaguenauerOdile Disciplina 571.96 Soggetti Genetic transformation Lingua di pubblicazione Inglese **Formato** Materiale a stampa Livello bibliografico Monografia Note generali Description based upon print version of record. Includes bibliographical references and index. Nota di bibliografia Nota di contenuto pt. 1. Technologies and pre-clinical studies -- pt. 2. Clinical trials and regulatory issues. Sommario/riassunto This book provides a 2012 reference for state-of-the-art gene transfer technology and the different aspects of its clinical translation with a focus on European-based initiatives. As examples of successful outcomes, recent clinical trials are presented together with Ethical, Safety and Regulatory issues, which are discussed. The broad range of various technologies is covered whether addressing direct in vivo gene transfer like with AAV, Adeno or non-viral vectors or ex-vivo genetically engineered cells including induced pluripotent stem cells (iPS) with integrating vectors such as retrovirus, lentivirus or transposon-derived systems. The critical path to clinical implementation is covered in the second part describing currently available tools - such as molecular imaging, ex-vivo organ cultures and high-throughput technologies used for evaluation of criteria towards a go-or-no go decision to move to the clinic; in addition, utmost salient biosafety and immunotoxicology aspects are discussed. This book is ideal for postgraduates, undergraduates, scientists, clinicians, regulators and patients' advocacy groups looking for states-of-the-art

information as well as emerging prospects - including gene targeting and homologous recombination - in gene transfer intended for clinical

translation.