BioSpectrum the business of Bio & Health Sciences

Gene therapy deals rise to 133 as FDA offers special designations

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Following Editas Medicine and AskBio's announcement of their strategic research collaboration to explore in-vivo delivery of genome editing medicines to treat neurological diseases;

Alessio Brunello, Senior Pharma Analyst at GlobalData, a leading data and analytics company, offers his view:"Editas Medicine and AskBio's collaboration comes to no surprise as several companies, especially in the biotech industry, are developing gene therapies. The market recoded 133 deals in 2018, as compared to 82 deals in 2014, representing a growth of about 62%.

"The translational gap to treatments based on gene therapy has been reduced in recent years because of improvements in gene editing tools such as the clustered regularly interspaced short palindromic repeats (CRISPR) with the CRISPR-associated protein (Cas) system and its variations. As in neurodegenerative diseases, this access is privileged – a system has allowed the development of more precise therapies for this disease area. However, before considering the clinical application of CRISPR/Cas, efficacy, safety, and delivery of the systems, need to be addressed due to the concerns regarding ethical issues in the research community.

"The high upfront costs associated with gene therapies make it difficult for patients to receive the curative care they need, thus restraining the innovation that brought the treatment to market.

"According to GlobalData, most preclinical and clinical gene therapies in development focus on extremely small patient population subsets or rare indications. The field has a number of historical failures, as it can be difficult to provide successful efficacy and safety data in the regenerative medicine space in general. Despite few setbacks and being a field typically associated with small specialist players, gene therapies have been gaining faster approvals lately, largely driven by special designations granted by the US Food and Drug Administration (FDA) - including breakthrough therapy designations and fast track designations - which allow manufacturers to pursue quicker pathways to market."