Literature Review

international level by EURORDIS and NORD in the US and other countries. The Rare Disease Day 2012 theme focused on solidarity, and the need for collaboration and mutual support in the field of rare diseases. Shire supported the Rare Disease Day 2012, a leading non-profit rare disease advocacy organization, in their Global Genes Project (Market Watch.htm). In India for the first time on February 28th, 2010 rare disease day was observed with support of LSD support society of India. In 2012, 29th February was observed as rare disease day and in India it was organised in Mumbai [2].

Tables 2 and 3 represent data on research and development being carried out in the field of rare diseases/orphan drugs and drugs which are in development pipeline respectively.

Methodology

Methodology includes systematic literature review with the aim to find out pharmaceutical companies which are leading at present in the rare disease market. The literature search revealed both MNC and biopharmaceutical companies are investing in developing drugs for rare diseases. Three companies Pfizer, Novartis and Shire, were identified which seems ahead of other companies in developing medicines and allocating resources for rare disease segment. A comparison between the numbers of drugs approved for these 3 companies, and the number of these drugs being awarded as orphan drugs status was made for the time period of 2007-2012. Further, to evaluate the trend of development of orphan drugs, the New Molecular Entities (NME) approved by US FDA as orphan drugs over a period of 6 years (2007-2012) were reviewed.

Results and Discussion

Figure 1 represents the total number of NME approved as orphan drugs over the period of 6 years (2007-2012) by USFDA. The numbers indicate that there is no clear trend in the number of NME approved drug status. In 2010, only 3 NME attained orphan drug status, whereas in 2011, 15 NME were approved as orphan drugs. The number again fell and only 6 NME were approved as orphan drugs by FDA in 2012. The comparative results of the 3 companies under study are represented in Figure 2. During the years 2007-2012, 75% of total approved drugs for Pfizer and Shire were granted orphan drug status. Pfizer's 6 out of total 8 approved drugs were granted orphan drug status, while Shire's 3 out of total 4 approved drugs were granted orphan drug status. Novartis

received USFDA approval for 21 drugs during this period and 10 drugs of these 21 were granted orphan drug status. As represented in Figure 1, there is no clear trend in the annual development of Orphan drugs but interestingly drugs approved for orphan diseases share a major part of the total number of drugs approved (as indicated in Figure 2, for 3 companies).

e three companies identi ed for this study have made a steady progress in recent years in the eld of rare diseases. Shire was one of the companies, along with Genzyme which started research in the area of rare diseases. But at present MNCs like P zer and Novartis have entered the orphan drug market and have acquired huge share in this earlier neglected eld. From 2009 onwards, big pharmaceutical companies account for 43% of total orphan drug approvals by FDA and claimed over 70% of market share, up from 56% in 2006, according to Business Communication Company (BCC) research (Market Forecasting). e success of P zer and Novartis as depicted in our study clearly indicates the growing interest of big pharmaceutical players in rare disease area. For getting the foothold in orphan disease area, MNC's have basically acquired small biotech companies involved in orphan drug development or have entered in a partnership with them [40]. Shire and Genzyme have Vipriv and Cerenzyme respectively in market for Gaucher treatment; despite this fact, P zer paid 60 million dollars to Protalixto acquire latter's Gaucher disease enzyme replacement therapy Taligurase Alfa [41]. P zer also acquired Fold Rx pharmaceuticals, whose main therapy area is developing drugs to treat diseases caused by protein misfolding [42]. Novartis is targeting orphan indications in cancer research. Its molecule Gleevec, rst got approval for Chronic Myelogenous Leukemia (CML) and subsequently Gleevec was granted Taligurase Alfa [41]. P zer also acquired Fold Rx pharmaceuticals,

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