


A BIRD'S-EYE VIEW OF THE RARE DISEASE LANDSCAPE

ORPHAN DRUG DEVELOPMENT TRENDS AND OPPORTUNITIES

MORE THAN
300 MILLION
PEOPLE
WORLDWIDE
HAVE A RARE
DISEASE


50% OF THE PEOPLE AFFECTED BY
RARE DISEASES ARE **CHILDREN**

~ **80%** RARE
DISEASES
ARE OF GENETIC ORIGIN


APPROVED TREATMENTS AVAILABLE
FOR ONLY **5%** OF ALL RARE DISEASES



FDA ORPHAN DRUG APPROVALS
> 500 SINCE THE PASSAGE OF THE
ORPHAN DRUG ACT

PROMISING
THERAPEUTIC
PIPELINE

560 RARE DISEASE DRUGS AND
THERAPIES IN DEVELOPMENT



~ **7,000**

DISEASES & DISORDERS
ARE CLASSIFIED AS RARE

IN THE LAST FIVE YEARS



OF ALL NEW DRUG APPROVALS
WERE FOR RARE DISEASES

TOP SELLING ORPHAN DRUGS

DRUG	COMPANY
REVLIMID	CELGENE
RITUXAN	ROCHE
COPAXONE	TEVA
OPDIVO	BMS
AVONEX	BIOGEN
IMBRUVICA	ABBVIE
SENSIPAR	AMGEN
GLEEVEC	NOVARTIS
VELCADE	TAKEDA
XYREM	JAZZ PHARMA

Orphan Diseases And Orphan Drugs

Roman Wölfel



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