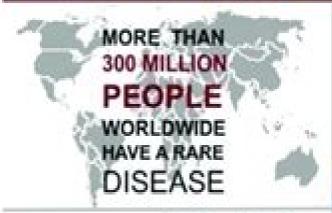
A BIRD'S-EYE VIEW OF THE RARE DISEASE LANDSCAPE ORPHAN DRUG DEVELOPMENT TRENDS AND OPPORTUNITIES





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 & DISORDERES ARE CLASSIFIED AS RARE

IN THE LAST FIVE YEARS



OF ALL NEW DRUG APPROVALS WERE FOR RARE DISEASES

TOP SELLING ORPHAN DRUGS

	101 OCCUPATION	DIG 10-01 DIG 00
	DRUG	COMPANY
	REVLIMID	CELGENE
	RITUXAN	ROCHE
	COPAXONE	TEVA
	OPDIVO	BMS
	AVONEX	BIOGEN
	IMBRUVICA	ABBVIE
	SENSIPAR	AMGEN
	GLEEVEC	NOVARTIS
	VELCADE	TAKEDA

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