CONGRESSIONAL BUDGET OFFICE
COST ESTIMATE

December 5, 2001

S. 1379
Rare Diseases Act of 2001

As ordered reported by the Senate Committee on Health, Education, Labor, and Pensions on November 1, 2001

SUMMARY

S. 1379 would provide for a statutory authorization of the Office of Rare Diseases (ORD) within the Office of the Director of the National Institutes of Health (NIH). The bill would authorize NIH to provide grants to regional "Centers of Excellence" to conduct research and training related to rare diseases and it would authorize funding for an existing grant program administered by the Food and Drug Administration (FDA) that sponsors clinical testing of the safety and effectiveness of new products to treat or diagnose rare diseases.

CBO estimates that implementing S. 1379 would cost $6 million in 2002 and $190 million over the 2002-2006 period, assuming the appropriation of the necessary amounts including annual adjustments for inflation. (The five-year total would be $181 million if such inflation adjustments are not made.) The legislation would not affect direct spending or receipts; therefore, pay-as-you-go procedures would not apply.

S. 1379 contains no intergovernmental or private sector mandates as defined in the Unfunded Mandates Reform Act (UMRA). State, local, and tribal governments could apply for and receive grants authorized by the bill, and any costs they incur would be voluntary.

ESTIMATED COST TO THE FEDERAL GOVERNMENT

The estimated budgetary impact of S. 1379 is shown in the following table. The costs of this legislation fall within budget function 550 (health).

	By Fiscal Year, in Millions of Dollars				
	2002	2003	2004	2005	2006
CHANGES IN SPENDING SUBJECT TO APPROPRIATION [a]					
Estimated Authorization Level					
National Institutes of Health [b]	24	25	25	26	27
Food and Drug Administration [c]	12	26	27	28	28
Total	36	51	52	54	55
Estimated Outlays					
National Institutes of Health	4	14	23	25	27
Food and Drug Administration	2	16	26	27	26
Total	6	30	49	52	53

a. The amounts shown reflect adjustments for anticipated inflation for those activities for which the bill would authorize such sums as necessary. Without such inflation adjustments, the five-year changes in authorization levels would total $232 million (instead of $248 million) and the changes in outlays would total $181 million (instead of $190 million).

b. The 2002-2006 levels are CBO baseline projections, including adjustments for anticipated inflation, for the NIH.

c. Estimated authorization level includes the incremental change from amounts appropriated thus far for 2002 for the FDA. The 2003-2006 levels are CBO baseline projections of the change in FDA authorization, including adjustments for anticipated inflation.

BASIS OF ESTIMATE

The bill would provide statutory authorization for the ORD, which was established in 1993 within the Office of the Director at the NIH. The ORD was established to respond to the reporting requirements of the Orphan Drug Act, to implement the recommendations of the National Commission on Orphan Diseases, and to respond to requests for information on rare diseases. A rare disease is one which typically affects fewer than 200,000 individuals in the United States.

The bill would require ORD to promote the establishment of a centralized clearinghouse to make data and information on rare diseases available to the public, researchers, and clinicians. The director of the ORD would be responsible for preparing biennial and annual reports on the activities of the office, its Centers of Excellence, and future research opportunities.

The bill would authorize NIH to award grants and contracts to public and nonprofit private entities known as Centers of Excellence to conduct clinical research, training, diagnostic, prevention, control, and or treatment activities with respect to rare diseases. The centers would be awarded renewable contracts for up to five years for each contract period.

S. 1379 would authorize appropriation of $24 million for those activities in 2002 and such sums as necessary for each subsequent year. CBO estimates that implementing the provisions affecting NIH would cost $2 million in 2002 and $97 million over the 2002-2006 period, assuming appropriation of the necessary amounts.

S. 1379 also would authorize funding for an existing grant program administered by FDA that sponsors clinical studies on the safety and effectiveness of new products to treat or diagnose rare diseases. The amount appropriated thus far for fiscal year 2002 for the current program is $13 million. The bill would authorize the appropriation of $25 million in 2002 and such sums as necessary for each subsequent fiscal year.

Research grants awarded under the program would defray some of the costs associated with clinical testing of certain orphan drugs, biologicals, medical devices, and medical foods. An orphan drug is a drug or biological that is used to treat or diagnose an illness usually affecting fewer than 200,000 people in the United States. Eligible medical devices and medical foods include products for which there is no reasonable expectation of development without grant assistance because the condition occurs relatively infrequently in the United States.

CBO estimates that implementing this provision for FDA grants would cost an additional $4 million in 2002 and $93 million over the 2002-2006 period, assuming appropriation of the necessary amounts.

PAY-AS-YOU-GO CONSIDERATIONS: None.

ESTIMATED IMPACT ON STATE, LOCAL, AND TRIBAL GOVERNMENTS

S. 1379 contains no intergovernmental mandates as defined in UMRA. State, local, and tribal governments could apply for and receive grants authorized by the bill, and any costs they incur would be voluntary.

ESTIMATED IMPACT ON THE PRIVATE SECTOR

The bill contains no private-sector mandates as defined in UMRA.

ESTIMATE PREPARED BY:

Federal Costs: Christopher J. Topoleski and Julia Christensen
Impact on State, Local, and Tribal Governments: Leo Lex
Impact on the Private Sector: Jennifer Bowman

ESTIMATE APPROVED BY:

Peter H. Fontaine
Deputy Assistant Director for Budget Analysis