Whereas, Congress passed the Orphan Drug Act (ODA) of 1983 in response to declining pharmaceutical investment of “orphaned” drugs through clinical trials following the Kefauver-Harris amendments of 1962 because of increased development costs;1,2,3 and

Whereas, The “orphan” designation is intended to incentivize the creation of drugs that target rare conditions affecting fewer than 200,000 Americans which are often deemed “unprofitable” due to the difficulty of recuperating development and marketing costs;4,5,6,7 and

Whereas, Although the ODA has been credited for introducing over 400 orphan drugs since becoming law, physicians, researchers, and policymakers have raised concerns about potential abuses of the Act;1-3,8,9,10 and

Whereas, Though the Act’s original intent was to incentivize the development of “non-profitable” therapies treating fewer than 200,000 Americans, several drugs have obtained “blockbuster” status indicating >$1 billion in sales annually, sometimes through a multitude of loopholes;1,4,9 and

Whereas, One such loophole is the approval for “orphan designation” - and therefore, ODA benefits - of existing compounds and mass-market drugs, as is the case for 3,4-DAP, ascorbic acid, calcium carbonate, Humira, and Crestor,8,11,12,13 and

Whereas, A pharmaceutical company may strategically submit a drug for approval of a single
indication - “one that is narrow enough to qualify for orphan drug benefits” - and once approved,
the drug is utilized for a variety of off-label uses, as demonstrated by the drugs rituximab,
modafinil, and a variety of oncology drugs;9,14,15 and

Whereas, The exploitation of loopholes within the Act have resulted in both exorbitant price
hikes and increasing sales, contributing up to one-fifth of global prescription sales by 2020
despite the original purpose of treating small populations;9,16,17 and

Whereas, Multiple pieces of legislation pertaining to the Orphan Drug Act have been submitted
by both parties in the 115th Congress, which along with recent action by the FDA, indicates
legislative and regulatory awareness of improvements that can be made and a will to do so;16,17
therefore be it

RESOLVED, That our American Medical Association support efforts to reform the Orphan Drug
Act by closing loopholes identified by the Food and Drug Administration in order to protect the
Act’s original intent of promoting therapies targeting rare diseases (New HOD Policy); and be it
further

RESOLVED, That our AMA support increased transparency in development costs, post-
approval regulation and overall earnings for pharmaceuticals designated as “Orphan Drugs”
(New HOD Policy); and be it further

RESOLVED, That our AMA support modifications to the exclusivity period of “Orphan Drugs” to
increase access to these pharmaceutical drugs for patients with rare diseases. (New HOD
Policy)

Fiscal Note: not yet determined

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RELEVANT POLICY:
Pharmaceutical Cost H-110.987
Cost of Prescription Drugs H-110.997
Cost of New Prescription Drugs H-110.998
Viability of Clinical Research Coverages and Reimbursement H-460.965

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15 Casali PG. The off-label use of drugs in oncology: a position paper by the European Society for Medical Oncology. Annals of
16 Gottlieb S. “FDA is advancing the goals of the Orphan Drug Act.” US Food & Drug Administration. Available at: