



Alzheimer's Disease / Effects on Federal Spending and Industry Innovation

Lauren Barnes, Director, provides strategic payer planning and public policy expertise to clients to assist in the successful commercialization of their products. She advises clients in the pharmaceutical and biotechnology industries.

Lauren was Director of the Payment and Coverage Group at Amgen, where she worked for six years. While at Amgen, Lauren held a series of leadership roles within its reimbursement and policy arenas. She led Amgen's internal payment policy and analysis team as well as Amgen's Medicaid and Medicare field teams. Lauren began her career at Amgen in its Washington, DC Government Affairs office. Immediately prior to her time with Amgen, Lauren worked for the Centers for Medicare and Medicaid Services (formerly HCFA) where she developed Medicare drug coverage policies within the Coverage and Analysis Group.

Lauren holds a B.A. in Public Health and an M.H.S. in Health Care Policy from The Johns Hopkins University.

Daniel Perry is the Executive Director of the not-for-profit Alliance for Aging Research in Washington, D.C. Founded in 1986, the Alliance is the nation's leading citizen advocacy organization for promoting a broad agenda of medical and scientific research to improve the health and independence of older Americans.

Mr. Perry's background spans a wide range of health policy, governmental, political and journalistic experience. Mr. Perry held staff positions for more than a dozen years on Capitol Hill, including special assistant to the Majority Whip of the U.S. Senate. He was appointed during the first Bush Administration to the Federal Task Force on Aging Research. He was also named by President Clinton to the Advisory Board of the White House Conference on Aging and served as a delegate to the 1995 and 2005 White House Conferences on Aging. He is chairman of the ACT-AD Coalition (Accelerate Cure/Treatments for Alzheimer's Disease) comprised of some 50 member organizations, as well as the multi-organization Friends of the National Institute on Aging.

Mr. Perry is past president and currently vice president and director of the Coalition for the Advancement of Medical Research (CAMR), leading over 100 U.S. patient groups, medical organizations and research universities in the fight to advance stem cell research and regenerative medicine. He is an advisor to the Institute on Aging of the University of Pennsylvania Medical School and a member of the New York Academy of Sciences.

Mr. Perry is a frequent speaker on aging research and public policy topics before business, academic, and public sector audiences, and is widely published on these subjects. As a journalist he was the recipient of many awards and citations, including a nomination for the Pulitzer Prize.

Janet Woodcock, M.D., Deputy Commissioner and the Chief Medical Officer, FDA. She oversees scientific and medical regulatory operations for FDA. Dr. Woodcock most recently served as the Deputy Commissioner for Operations and Chief Operating Officer, FDA 2005-2007. Dr. Woodcock served as Director, Center for Drug Evaluation and Research at FDA 1994-2005. She previously served in other positions at FDA including Director, Office of Therapeutics Research and Review and Acting Deputy Director, Center for Biologics Evaluation and Research. Dr. Woodcock received her M.D. from Northwestern Medical School, and completed further training and held teaching appointments at the Pennsylvania State University and the University of California in San Francisco. She joined FDA in 1986.



Comparative Analysis of FDA Review Times for Alzheimer's, HIV/AIDS, and Cancer Therapies

July 25, 2007

Lauren Barnes
Avalere Health LLC

Alzheimer's Disease (Alzheimer's) Poses a Unique and Burgeoning Threat to the U.S. Public Health System

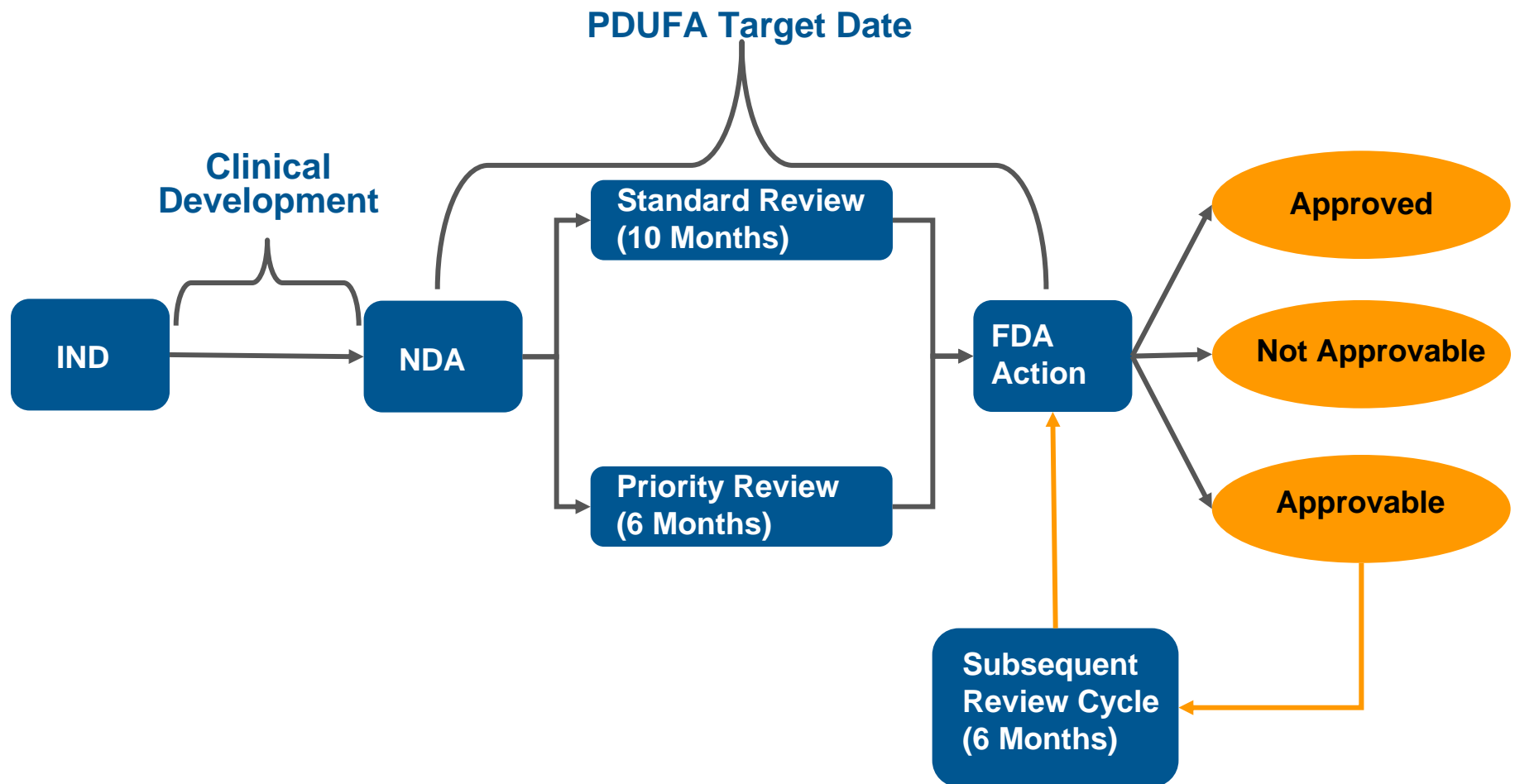
- Age is the number one risk factor
 - » Steady aging of the Baby Boomers will increase the impact
- Alzheimer's is a key determinant of needing long-term care
 - » Unpaid or donated care
 - » Individual, out-of-pocket payment
 - » Medicare and Medicaid
 - » No wide-spread insurance coverage of long-term care costs
- Alzheimer's places a tremendous burden on families

Current Alzheimer's Treatments Offer Symptomatic Relief, But No Disease Modification

- Speed of patient access to new treatments depends on several critical factors
 - » Medical innovation
 - » FDA* review process
- PDUFA* passed to expedite the review process
 - » Set target timelines for review of an NDA or BLA*
 - » Timelines have decreased over time
 - » PDUFA appears to have markedly improved FDA review time

* FDA = Food and Drug Administration. PDUFA = Prescription Drug User Fee Act. NDA = New Drug Application. BLA = Biologic License Application.

PDUFA Also Served to Affect Time to Market in Other Areas That Are Not Measured or Reported Publicly by the FDA



Avalere's Study Assessed FDA Review Times for Alzheimer's Therapies as Compared to Other Diseases

- Review time for Alzheimer's drugs compared to other life threatening diseases :
 - » HIV/AIDS
 - » Prostate, Ovarian, and Breast Cancers
 - Life threatening and likely to affect the elderly
- No single repository of data or abundance of public records
 - » Avalere reviewed a number of databases
 - » Considered only FDA review times per product (no clinical differences)
 - » Analysis included obtainment of PDUFA timelines
 - Priority Review and first-cycle approval also studied

* HIV/AIDS = Human Immunodeficiency Virus/Acquired Immune Deficiency Syndrome.

FDA Met PDUFA Review Times for Analyzed Products

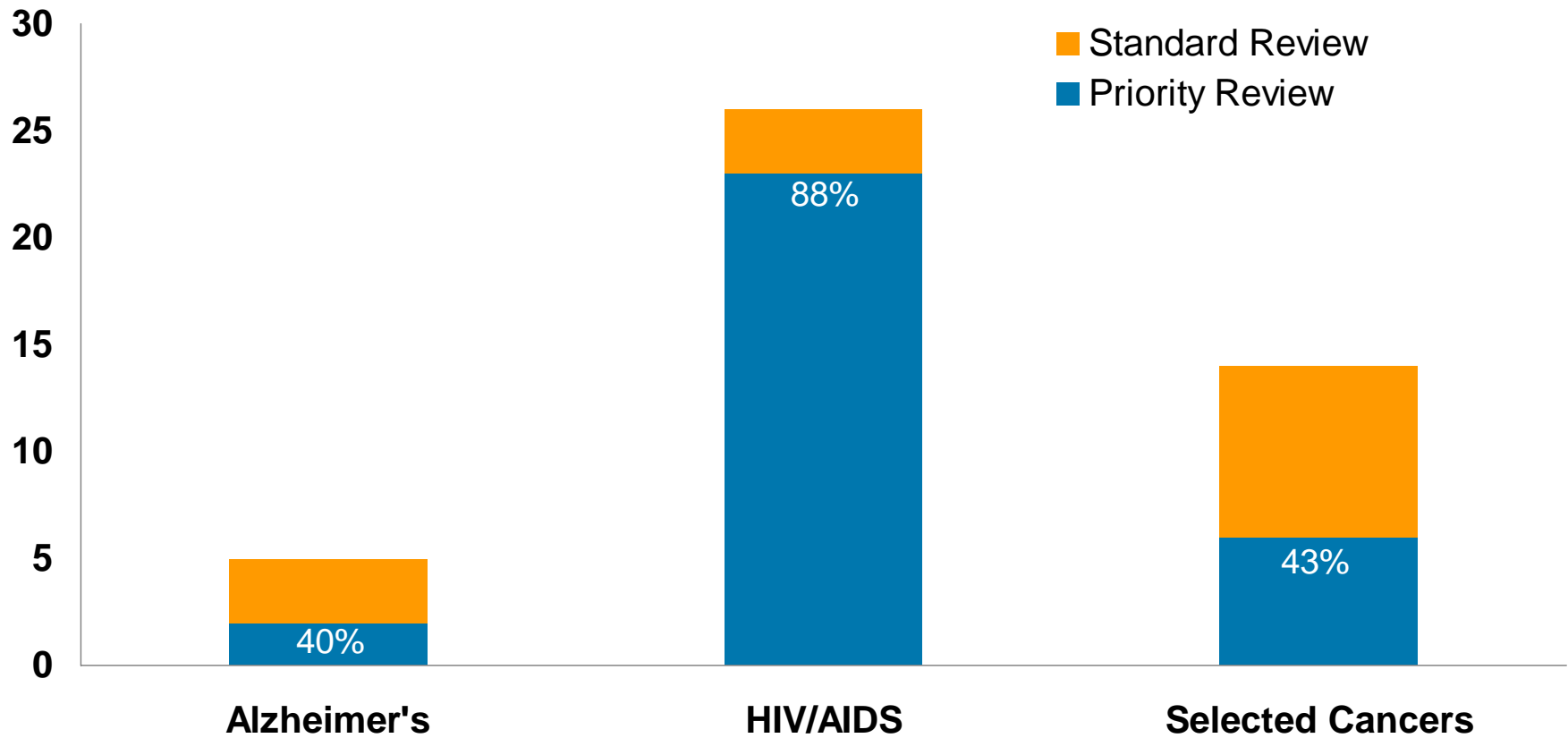
Disease State	Products Analyzed	Notes
Alzheimer's	5	<ul style="list-style-type: none">All approved products analyzedAll approved products are for symptom relief*
HIV/AIDS	26	<ul style="list-style-type: none">All approved products analyzedDrugs were stratified by class for analysis (i.e., entry inhibitors, NRTIs, NNRTIs, and protease inhibitors)**
Cancer	18	<ul style="list-style-type: none">All approved products analyzed where specified cancer was the <u>initial</u> indicationDrugs were stratified by indication for analysis (i.e., prostate, ovarian, breast)

FDA met PDUFA review timelines for all drug categories; there was no evidence of a slower or delayed FDA review time for Alzheimer's.

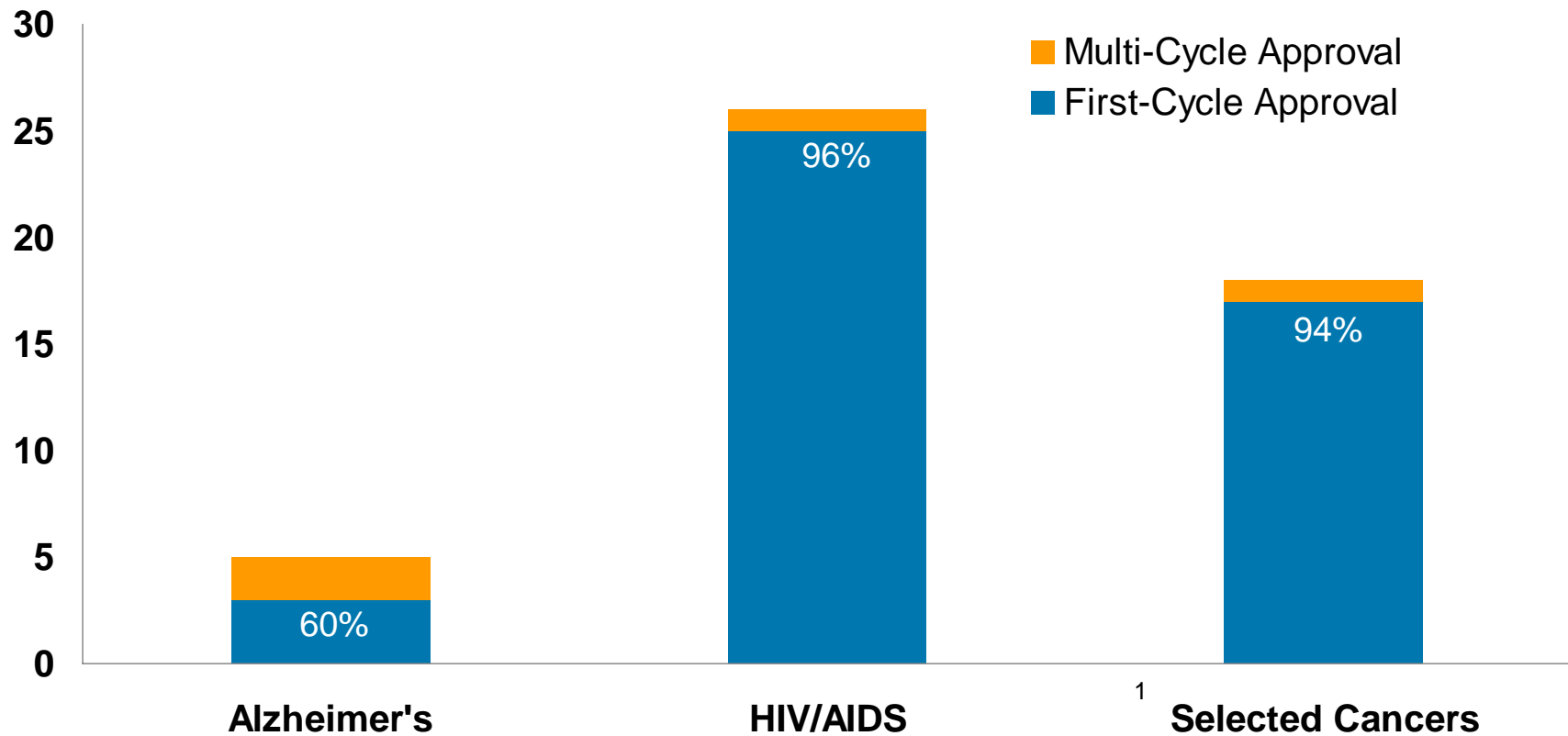
* Disease-modifying agents in development were analyzed, but there were insufficient data for conclusions.

** NRTI = nucleoside reverse transcriptase inhibitor. NNRTI = non-nucleoside reverse transcriptase inhibitor.

HIV/AIDS Drugs Obtain Priority Review Status More Often than Comparator Disease States



Cancer and HIV/AIDS Products Obtain First-Cycle Approval More Frequently than Alzheimer's Drugs



Avalere Study Showed Potential Differences in Speed to Market for Alzheimer's Products Compared to Other Therapies

- The FDA met its target goals with respect to Alzheimer's
 - » However, HIV/AIDS and cancer drugs *exceeded* these targets
- Many possible explanations for these findings
 - » Small sample size
 - » FDA division and FDA reviewer per product
 - » Clinical complexity of the individual product
 - » Strength and completeness of initial FDA submission
 - » Changing public perception of a particular disease
 - » Increased scientific or social understanding of a particular disease

A deeper review of the FDA review process and any potential barriers can help policymakers, industry, patients, and their advocates work together to make improvements for future evaluation of emerging medical technology.

Alzheimer's Disease and its Effect on Federal Spending and Industry Innovation

July 25, 2007

Daniel Perry, Chair

ACT-AD

Accelerate Cure/Treatments for Alzheimer's Disease

ACT-AD

- **Accelerate Cure/Treatments for Alzheimer's Disease**
- **Coalition of 50+ national organizations representing patients, providers, caregivers, consumers, older Americans, researchers, and employers seeking to accelerate development of potential cures and treatments for Alzheimer's disease**
- **www.act-ad.org**

ACT-AD

Goals:

- **Generate immediate public and government recognition of Alzheimer's disease as a debilitating, dehumanizing and life-threatening disease that requires urgent attention**
- **Bring transformational therapies to patients, providers and families in the next decade by making the acceleration of promising Alzheimer's therapies a top national priority**

Updates on the Scale of the Alzheimer's Epidemic

- **According to new figures from the Alzheimer's Association:**
 - ▶ More than 5 million Americans have Alzheimer's today
 - ▶ More than a 50% increase in cases by 2030
 - ▶ 16 million by 2050
- **The Lewin Group 2004 study on cost of Alzheimer's care:**
 - ▶ State and federal Medicaid spending on nursing home care alone in 2004: \$19 billion
 - ▶ By 2025, almost \$38 billion
 - ▶ By 2050, \$54 billion

The Treatment Horizon

- **120 compounds currently in development with potential to delay or reverse onset of Alzheimer's**
- **Almost two dozen treatments in late-stage development**
- **Currently available therapies that change the course of the disease: 0**

Progress on Alzheimer's Treatments

Food and Drug Administration

- **Creation of Intra-agency Neurology Working Group**
 - ▶ Expand FDA awareness of leading-edge development
 - ▶ Enable sharing of technical and regulatory expertise
 - ▶ Provide for greater consistency of review standards and processes across FDA
- **Expanding Patient Representative/Consultant Program to include AD**
 - ▶ AD advocates advise FDA on new medical products
 - ▶ Participate in FDA Advisory Committee Meetings

Progress on Alzheimer's Treatments

- **Series of hearings by Senator Mikulski**
- **Ongoing advocacy for increased appropriations for FDA**
- **Non-partisan Alzheimer's Disease Study Group**

ACT-AD Research Reports

- **Comparison of FDA's Review of Several Categories of Drugs including AD**
 - ▶ Avalere Health
- **Alzheimer's Disease and Cost-effectiveness Analyses: Ensuring Good Value for Money?**
 - ▶ University of Conn./Center for Medicine in the Public Interest

Alzheimer's Disease and Cost-effectiveness Analyses: Ensuring Good Value for Money

- **A QALY- quality adjusted life year: An additional year of life measured not just in terms of actual survival but in terms of quality of life**
- **The current QALY amount of \$50,000 is antiquated**

Why the New Formula?

- **QALY in the United States has been significantly underestimated**
- **Recent estimates in the U.S. of the value of a life year are closer to \$175,000**
 - ▶ Murphy and Topel/University of Chicago

Recalculating the Value of Delaying Alzheimer's

- **\$175,000 per QALY**
- **Without treatment breakthroughs, 16 million people with Alzheimer's by 2050**
- **Promising drugs are currently in development or FDA review**

Recalculating the Value of Delaying Alzheimer's

AD Drug Effectiveness	Delay AD Onset by 1 Year	Delay AD Onset by 3 Years	Delay AD Onset by 5 Years
QALY Gains	6.86 Million	17.29 Million	22.66 Million
Dollar Value (\$175,000/QALY)	\$1.20 trillion	\$3.03 trillion	\$3.97 trillion

ACT-AD

Future Plans:

- **Recruit patients and caregivers for FDA consultant/representative program**
- **Ally meetings with FDA**
- **Continue to identify gaps and sponsor research**
- **Continue to build awareness, call for action to accelerate access to new therapies**

Thank You

ACT-AD

Accelerate Cure/Treatments for Alzheimer's Disease

www.act-ad.org