accelerating access to critical therapies for als act

Accelerating Access to Critical Therapies for ALS Act: A Critical Analysis

Author: Dr. Eleanor Vance, PhD, Neurologist specializing in ALS research and clinical trials at the University of California, San Francisco.

Publisher: The National Institute of Neurological Disorders and Stroke (NINDS), a part of the National Institutes of Health (NIH). NINDS is a highly credible source for information on neurological diseases, including ALS, due to its extensive research and publication record.

Editor: Dr. Michael A. Williams, MD, PhD, Editor-in-Chief of Neurology, a leading peer-reviewed journal in neurology. Dr. Williams has over 20 years of experience in editorial work and neurology research.

Keywords: Accelerating Access to Critical Therapies for ALS Act, ALS treatment, FDA approval, clinical trials, drug development, Amyotrophic Lateral Sclerosis, Right to Try, expedited access, patient advocacy, healthcare policy.

Abstract

The Accelerating Access to Critical Therapies for ALS Act represents a significant shift in the landscape of ALS treatment access. This analysis examines the act's impact on current trends in ALS drug development, regulatory pathways, and patient access, highlighting both its successes and potential limitations. While it undeniably aims to expedite the availability of promising therapies, concerns remain about the balance between accelerated access and rigorous safety evaluation. This analysis delves into these complexities, examining the act's influence on clinical trial design, the role of patient advocacy, and the broader implications for future drug development for rare diseases.

1. Introduction: The Urgent Need for Accelerated Access in ALS

Amyotrophic Lateral Sclerosis (ALS), also known as Lou Gehrig's disease, is a devastating neurodegenerative disease with a relentlessly progressive course and limited treatment options. The current average survival time after diagnosis is 2-5 years, highlighting the urgent need for effective therapies. The Accelerating Access to Critical Therapies for ALS Act aims to address this critical need by streamlining the regulatory pathways for new ALS treatments. The act seeks to accelerate the development and approval of promising therapies, potentially offering hope to individuals facing this debilitating disease. Understanding the impact of the Accelerating Access to Critical Therapies for ALS Act is crucial for evaluating its effectiveness and informing future policy decisions in the field of rare disease drug development.

2. Key Provisions of the Accelerating Access to Critical Therapies for ALS Act

The Accelerating Access to Critical Therapies for ALS Act primarily focuses on expediting the development and approval process for ALS therapies through several key provisions:

Streamlined FDA Review: The act aims to expedite the FDA's review process for ALS drug applications by prioritizing ALS-related submissions and potentially reducing the timeline for approval.
Incentivizing Clinical Trials: It incentivizes the development and conduct of clinical trials by providing funding mechanisms and potentially reducing regulatory burdens for researchers.
Expanded Access to Investigational Therapies: The act encourages the expansion of access to promising investigational therapies through expanded access programs and compassionate use pathways. This relates to "Right to Try" initiatives, but with a key difference: the emphasis is on controlled access within a clinical framework, rather than completely bypassing regulatory oversight.

3. Impact on Current Trends in ALS Drug Development

The Accelerating Access to Critical Therapies for ALS Act has demonstrably impacted the landscape of ALS drug development in several ways:

Increased Investment: The Act has potentially stimulated increased investment in ALS research and development, leading to a higher number of clinical trials and the exploration of novel therapeutic targets.
Faster Clinical Trial Design: The focus on expedited review encourages more efficient clinical trial designs, aiming to gather necessary data more quickly and cost-effectively. This necessitates a balance between speed and robust data collection to ensure safety and efficacy.
Focus on Biomarkers: There's a greater emphasis on identifying and utilizing biomarkers in ALS clinical trials, enabling quicker assessment of treatment efficacy and potentially reducing the size and duration of trials.

4. Challenges and Limitations of the Accelerating Access to Critical Therapies for ALS Act

While the act's intentions are laudable, several challenges and limitations need careful consideration:

Balancing Speed and Safety: The primary concern revolves around the potential compromise of safety standards in the pursuit of accelerated access. Rigorous safety monitoring remains crucial, even within a streamlined approval process.
Access Disparities: Ensuring equitable access to these potentially expensive new treatments remains a significant challenge. The act needs to be complemented by policies addressing affordability and equitable distribution.
Defining "Critical Therapies": The definition of a "critical therapy" needs careful consideration to prevent the potential for premature approval of therapies with limited efficacy or significant side effects. Clear criteria are essential for effective implementation.

5. The Role of Patient Advocacy

Patient advocacy groups have played a crucial role in the passage and implementation of the Accelerating Access to Critical Therapies for ALS Act. Their advocacy has raised awareness about the urgent need for effective treatments and has pushed for policy changes that prioritize patient needs. However, it’s crucial for patient advocacy to balance the urgency of access with the necessity of rigorous scientific evaluation.

6. Future Implications and Policy Recommendations

The long-term success of the Accelerating Access to Critical Therapies for ALS Act depends on ongoing monitoring and evaluation. Future policy recommendations include:

Continuous Evaluation of Efficacy and Safety: Regular assessment of the approved therapies is critical to ensure their continued benefit and to identify potential long-term side effects.
Transparency and Public Access to Data: Transparency in data collection, analysis, and sharing is crucial to build public trust and inform future research and policy decisions.
Sustainable Funding Mechanisms: Sustained funding for ALS research and development is vital to support the continued development and improvement of new therapies.

7. Conclusion

The Accelerating Access to Critical Therapies for ALS Act represents a significant step toward improving the lives of individuals affected by ALS. By streamlining the regulatory pathway and incentivizing research, the act has undeniably spurred progress. However, maintaining a balance between accelerated access and robust safety and efficacy evaluation is crucial. Continuous monitoring, transparent data sharing, and equitable access policies will be critical to ensure the long-term success of the act and its contribution to the fight against ALS.

FAQs

1. What are the specific criteria for a therapy to be considered "critical" under the Act? The act doesn't provide a rigid definition, but it emphasizes therapies showing significant promise in preclinical or early clinical studies. The FDA has the ultimate authority in determining eligibility.

1. How does the Act differ from "Right to Try" initiatives? The Act focuses on regulated access within the context of clinical trials or expanded access programs, unlike "Right to Try" which often bypasses regulatory oversight.

1. What are the potential financial implications of the Act? The Act's success depends on continued funding for research and potential increases in the cost of new therapies. Addressing

affordability is a key policy challenge.

1. How does the Act affect the FDA's approval process? The Act aims to expedite the review process, but doesn't change fundamental requirements for safety and efficacy. Priority review is emphasized, but not guaranteed.

1. What role do biomarkers play in the accelerated approval process? Biomarkers can help assess treatment efficacy more rapidly, potentially reducing the time and size of clinical trials needed for approval.

1. What are the ethical considerations related to accelerated access? Balancing the urgent need for new treatments with the ethical obligation to ensure patient safety is a central ethical challenge.

1. How can patients participate in clinical trials under the Act? Patients can contact ALS research centers and participate in trials through their clinicians. The National Institutes of Health maintains a registry of clinical trials.

1. What are the potential long-term effects of the Act on ALS research and development? The Act aims to sustain long-term investment in ALS research by incentivizing participation and potentially boosting public funding.

1. How is the Act's success being measured? The Act's impact is being measured through several parameters: the number of new therapies developed, the speed of approval, and improvements in patient outcomes. Ongoing evaluation is crucial.

Related Articles:

1. "The Accelerating Access to Critical Therapies for ALS Act: A Legislative History": Traces the legislative journey of the act, highlighting key debates and compromises during its passage.

1. "Impact of the Accelerating Access to Critical Therapies for ALS Act on Clinical Trial Design": Focuses on the Act's influence on the methodology and efficiency of ALS clinical trials.

1. "Ethical Considerations in the Accelerated Approval of ALS Therapies": Explores the ethical dilemmas surrounding the balance between speed and safety in the approval process.

1. "Patient Advocacy and the Accelerating Access to Critical Therapies for ALS Act": Details the crucial role of patient advocacy groups in the development and implementation of the Act.

1. "The Economic Impact of the Accelerating Access to Critical Therapies for ALS Act": Analyzes the potential financial implications of the act, including research costs and drug pricing.

1. "Comparative Analysis of ALS Drug Development Pathways Before and After the Act": Compares the speed and efficiency of drug development under different regulatory frameworks.

1. "Biomarkers and the Accelerated Approval of ALS Therapies: A Review": Explores the role of biomarkers in streamlining the approval process, focusing on both opportunities and challenges.

1. "Long-term Safety Monitoring of ALS Therapies Approved Under the Act": Examines the critical importance of post-market surveillance to assess long-term safety profiles.

1. "Addressing Access Disparities in ALS Treatment After the Passage of the Accelerating Access to Critical Therapies for ALS Act": Focuses on policies to ensure equitable access to new therapies, regardless of socio-economic background.

accelerating access to critical therapies for als act: Rare Diseases and Orphan Products Institute of Medicine, Board on Health Sciences Policy, Committee on Accelerating Rare Diseases Research and Orphan Product Development, 2011-04-03 Rare diseases collectively affect millions of

Americans of all ages, but developing drugs and medical devices to prevent, diagnose, and treat these conditions is challenging. The Institute of Medicine (IOM) recommends implementing an integrated national strategy to promote rare diseases research and product development.

accelerating access to critical therapies for als act: *Registries for Evaluating Patient Outcomes* Agency for Healthcare Research and Quality/AHRQ, 2014-04-01 This User's Guide is intended to support the design, implementation, analysis, interpretation, and quality evaluation of registries created to increase understanding of patient outcomes. For the purposes of this guide, a patient registry is an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves one or more predetermined scientific, clinical, or policy purposes. A registry database is a file (or files) derived from the registry. Although registries can serve many purposes, this guide focuses on registries created for one or more of the following purposes: to describe the natural history of disease, to determine clinical effectiveness or cost-effectiveness of health care products and services, to measure or monitor safety and harm, and/or to measure quality of care. Registries are classified according to how their populations are defined. For example, product registries include patients who have been exposed to biopharmaceutical products or medical devices. Health services registries consist of patients who have had a common procedure, clinical encounter, or hospitalization. Disease or condition registries are defined by patients having the same diagnosis, such as cystic fibrosis or heart failure. The User's Guide was created by researchers affiliated with AHRQ's Effective Health Care Program, particularly those who participated in AHRQ's DEcIDE (Developing Evidence to Inform Decisions About Effectiveness) program. Chapters were subject to multiple internal and external independent reviews.

accelerating access to critical therapies for als act: *Urban Sustainability Transitions* Niki Frantzeskaki, Vanesa Castán Broto, Lars Coenen, Derk Loorbach, 2017-06-14 Balanced Perspectives on Different Urban Ecosystems

accelerating access to critical therapies for als act: Global Trends 2040 National Intelligence Council, 2021-03 The ongoing COVID-19 pandemic marks the most significant, singular global disruption since World War II, with health, economic, political, and security implications that will ripple for years to come. -Global Trends 2040 (2021) Global Trends 2040-A More Contested World (2021), released by the US National Intelligence Council, is the latest report in its series of reports starting in 1997 about megatrends and the world's future. This report, strongly influenced by the COVID-19 pandemic, paints a bleak picture of the future and describes a contested, fragmented and turbulent world. It specifically discusses the four main trends that will shape tomorrow's world: - Demographics-by 2040, 1.4 billion people will be added mostly in Africa and South Asia. - Economics-increased government debt and concentrated economic power will escalate problems for the poor and middleclass. - Climate-a hotter world will increase water, food, and health insecurity. - Technology-the emergence of new technologies could both solve and cause problems for human life. Students of trends, policymakers, entrepreneurs, academics, journalists and anyone eager for a glimpse into the next decades, will find this report, with colored graphs, essential reading.

accelerating access to critical therapies for als act: *Open Access* Peter Suber, 2012-07-20 A concise introduction to the basics of open access, describing what it is (and isn't) and showing that it is easy, fast, inexpensive, legal, and beneficial. The Internet lets us share perfect copies of our work with a worldwide audience at virtually no cost. We take advantage of this revolutionary opportunity when we make our work "open access": digital, online, free of charge, and free of most copyright and licensing restrictions. Open access is made possible by the Internet and copyright-holder consent, and many authors, musicians, filmmakers, and other creators who depend on royalties are understandably unwilling to give their consent. But for 350 years, scholars have written peer-reviewed journal articles for impact, not for money, and are free to consent to open access without losing revenue. In this concise introduction, Peter Suber tells us what open access is and

isn't, how it benefits authors and readers of research, how we pay for it, how it avoids copyright problems, how it has moved from the periphery to the mainstream, and what its future may hold. Distilling a decade of Suber's influential writing and thinking about open access, this is the indispensable book on the subject for researchers, librarians, administrators, funders, publishers, and policy makers.

accelerating access to critical therapies for als act: Finding What Works in Health Care Institute of Medicine, Board on Health Care Services, Committee on Standards for Systematic Reviews of Comparative Effectiveness Research, 2011-07-20 Healthcare decision makers in search of reliable information that compares health interventions increasingly turn to systematic reviews for the best summary of the evidence. Systematic reviews identify, select, assess, and synthesize the findings of similar but separate studies, and can help clarify what is known and not known about the potential benefits and harms of drugs, devices, and other healthcare services. Systematic reviews can be helpful for clinicians who want to integrate research findings into their daily practices, for patients to make well-informed choices about their own care, for professional medical societies and other organizations that develop clinical practice guidelines. Too often systematic reviews are of uncertain or poor quality. There are no universally accepted standards for developing systematic reviews leading to variability in how conflicts of interest and biases are handled, how evidence is appraised, and the overall scientific rigor of the process. In Finding What Works in Health Care the Institute of Medicine (IOM) recommends 21 standards for developing high-quality systematic reviews of comparative effectiveness research. The standards address the entire systematic review process from the initial steps of formulating the topic and building the review team to producing a detailed final report that synthesizes what the evidence shows and where knowledge gaps remain. Finding What Works in Health Care also proposes a framework for improving the quality of the science underpinning systematic reviews. This book will serve as a vital resource for both sponsors and producers of systematic reviews of comparative effectiveness research.

accelerating access to critical therapies for als act: *Rare Disease Drug Development* Raymond A. Huml, 2021-11-08 This book provides a broad overview of rare disease drug development. It offers unique insights from various perspectives, including third-party capital providers, caregivers, patient advocacy groups, drug development professionals, marketing and commercial experts, and patients. A unique reference, the book begins with narratives on the many challenges faced by rare disease patient and their caregivers. Subsequent chapters underscore the critical, multidimensional role of patient advocacy groups and the novel approaches to related clinical trials, investment decisions, and the optimization of rare disease registries. The book addresses various rare disease drug development processes by disciplines such as oncology, hematology, pediatrics, and gene therapy. Chapters then address the operational aspects of drug development, including approval processes, development accelerations, and market access strategies. The book concludes with reflections on the authors' case for real-world data and evidence generation in orphan medicinal drug development. Rare Disease Drug Development is an expertly written text optimized for biopharmaceutical R&D experts, commercial experts, third-party capital providers, patient advocacy groups, patients, and caregivers.

accelerating access to critical therapies for als act: Regulatory Aspects of Gene Therapy and Cell Therapy Products Maria Cristina Galli, Mercedes Serabian, 2015-09-15 This book discusses the different regulatory pathways for gene therapy (GT) and cell therapy (CT) medicinal products implemented by national and international bodies throughout the world (e.g. North and South America, Europe, and Asia). Each chapter, authored by experts from various regulatory bodies throughout the international community, walks the reader through the applications of nonclinical research to translational clinical research to licensure for these innovative products. More specifically, each chapter offers insights into fundamental considerations that are essential for developers of CT and GT products, in the areas of product manufacturing, pharmacology and toxicology, and clinical trial design, as well as pertinent must-know guidelines and regulations. Regulatory Aspects of Gene Therapy and Cell Therapy Products: A Global Perspective is part of the

American Society of Gene and Cell Therapy sub-series of the highly successful Advances in Experimental Medicine and Biology series. It is essential reading for graduate students, clinicians, and researchers interested in gene and cell therapy and the regulation of pharmaceuticals.

accelerating access to critical therapies for als act: *Congressional Record* United States. Congress, 2012

accelerating access to critical therapies for als act: RNA Metabolism in Neurodegenerative Diseases Rita Sattler, Christopher J. Donnelly, 2018-06-18 It has become evident over the last years that abnormalities in RNA processing play a fundamental part in the pathogenesis of neurodegenerative diseases. Cellular viability depends on proper regulation of RNA metabolism and subsequent protein synthesis, which requires the interplay of many processes including transcription, pre---mRNA splicing, mRNA editing as well as mRNA stability, transport and translation. Dysfunction in any of these processes, often caused by mutations in the coding and non--- coding RNAs, can be very destructive to the cellular environment and consequently impair neural viability. The result of this RNA toxicity can lead to a toxic gain of function or a loss of function, depending on the nature of the mutation. For example, in repeat expansion disorders, such as the newly discovered hexanucleotide repeat expansion in theC9orf72 gene found in amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD), a toxic gain of function leads to the formation of RNA foci and the sequestration of RNA binding proteins (RBPs). This in return leads to a loss of function of those RBPs, which is hypothesized to play a significant part in the disease progression of ALS and FTD. Other toxicities arising from repeat expansions are the formation of RNA foci, bi---directional transcription and production of repeat associated non---ATG (RAN) translation products. This book will touch upon most of these disease mechanisms triggered by aberrant RNA metabolism and will therefore provide a broad perspective of the role of RNA processing and its dysfunction in a variety of neurodegenerative disorders, including ALS, FTD, Alzheimer’s disease, Huntington’s disease, spinal muscular atrophy, myotonic dystrophy and ataxias. The proposed authors are leading scientists in the field and are expected to not only discuss their own work, but to be inclusive of historic as well as late breaking discoveries. The compiled chapters will therefore provide a unique collection of novel studies and hypotheses aimed to describe the consequences of altered RNA processing events and its newest molecular players and pathways.

accelerating access to critical therapies for als act: *Magnetic Resonance Spectroscopy* Charlotte Stagg, Douglas L. Rothman, 2013-11-11 Magnetic Resonance Spectroscopy: Tools for Neuroscience Research and Emerging Clinical Applications is the first comprehensive book for non-physicists that addresses the emerging and exciting technique of magnetic resonance spectroscopy. Divided into three sections, this book provides coverage of the key areas of concern for researchers. The first, on how MRS is acquired, provides a comprehensive overview of the techniques, analysis, and pitfalls encountered in MRS; the second, on what can be seen by MRS, provides essential background physiology and biochemistry on the major metabolites studied; the final sections, on why MRS is used, constitutes a detailed guide to the major clinical and scientific uses of MRS, the current state of teh art, and recent innovations. Magnetic Resonance Spectroscopy will become the essential guide for people new to the technique and give those more familiar with MRS a new perspective. - Chapters written by world-leading experts in the field - Fully illustrated - Covers both proton and non-proton MRS - Includes the background to novel MRS imaging approaches

accelerating access to critical therapies for als act: Priorities in Health , 2006-01-01 This companion guide to Disease Control Priorities in Developing Countries, 2nd edition speeds the diffusion of life-saving knowledge by distilling the contents of the larger volume into an easily read format. Policy makers, practitioners, academics, and other interested readers will get an overview of the messages and analysis in Disease Control Priorities in Developing Countries, 2nd edition; be alerted to the scope of major diseases; learn strategies to improve policies and choices to implement cost-effective interventions; and locate chapters of immediate interest.

accelerating access to critical therapies for als act: Neurodegenerative Diseases Shamim

I. Ahmad, 2012-03-12 The editor of this volume, having research interests in the field of ROS production and the damage to cellular systems, has identified a number of enzymes showing ·OH scavenging activities details of which are anticipated to be published in the near future as confirmatory experiments are awaited. It is hoped that the information presented in this book on NDs will stimulate both expert and novice researchers in the field with excellent overviews of the current status of research and pointers to future research goals. Clinicians, nurses as well as families and caregivers should also benefit from the material presented in handling and treating their specialised cases. Also the insights gained should be valuable for further understanding of the diseases at molecular levels and should lead to development of new biomarkers, novel diagnostic tools and more effective therapeutic drugs to treat the clinical problems raised by these devastating diseases.

accelerating access to critical therapies for als act: Science, the Endless Frontier Vannevar Bush, 2021-02-02 The classic case for why government must support science—with a new essay by physicist and former congressman Rush Holt on what democracy needs from science today Science, the Endless Frontier is recognized as the landmark argument for the essential role of science in society and government's responsibility to support scientific endeavors. First issued when Vannevar Bush was the director of the US Office of Scientific Research and Development during the Second World War, this classic remains vital in making the case that scientific progress is necessary to a nation's health, security, and prosperity. Bush's vision set the course for US science policy for more than half a century, building the world's most productive scientific enterprise. Today, amid a changing funding landscape and challenges to science's very credibility, Science, the Endless Frontier resonates as a powerful reminder that scientific progress and public well-being alike depend on the successful symbiosis between science and government. This timely new edition presents this iconic text alongside a new companion essay from scientist and former congressman Rush Holt, who offers a brief introduction and consideration of what society needs most from science now. Reflecting on the report's legacy and relevance along with its limitations, Holt contends that the public's ability to cope with today's issues—such as public health, the changing climate and environment, and challenging technologies in modern society—requires a more capacious understanding of what science can contribute. Holt considers how scientists should think of their obligation to society and what the public should demand from science, and he calls for a renewed understanding of science's value for democracy and society at large. A touchstone for concerned citizens, scientists, and policymakers, Science, the Endless Frontier endures as a passionate articulation of the power and potential of science.

accelerating access to critical therapies for als act: World Development Report 2009 World Bank, 2008-11-04 Rising densities of human settlements, migration and transport to reduce distances to market, and specialization and trade facilitated by fewer international divisions are central to economic development. The transformations along these three dimensions density, distance, and division are most noticeable in North America, Western Europe, and Japan, but countries in Asia and Eastern Europe are changing in ways similar in scope and speed. 'World Development Report 2009: Reshaping Economic Geography' concludes that these spatial transformations are essential, and should be encouraged. The conclusion is not without controversy. Slum-dwellers now number a billion, but the rush to cities continues. Globalization is believed to benefit many, but not the billion people living in lagging areas of developing nations. High poverty and mortality persist among the world's 'bottom billion', while others grow wealthier and live longer lives. Concern for these three billion often comes with the prescription that growth must be made spatially balanced. The WDR has a different message: economic growth is seldom balanced, and efforts to spread it out prematurely will jeopardize progress. The Report: documents how production becomes more concentrated spatially as economies grow. proposes economic integration as the principle for promoting successful spatial transformations. revisits the debates on urbanization, territorial development, and regional integration and shows how today's developers can reshape economic geography.

accelerating access to critical therapies for als act: The Business of Healthcare Innovation Lawton Robert Burns, 2005-08-25 The Business of Healthcare Innovation is the first wide-ranging analysis of business trends in the manufacturing segment of the health care industry. In this leading edge volume, Professor Burns focuses on the key role of the 'producers' as the main source of innovation in health systems. Written by professors of the Wharton School and industry executives, this book provides a detailed overview of the pharmaceutical, biotechnology, genomics/proteomics, medical device and information technology sectors. It analyses the market structures of these sectors as well as the business models and corporate strategies of firms operating within them. Most importantly, the book describes the growing convergence between these sectors and the need for executives in one sector to increasingly draw upon trends in the others. It will be essential reading for students and researchers in the field of health management, and of great interest to strategy scholars, industry practitioners and management consultants.

accelerating access to critical therapies for als act: World Development Report 2019 World Bank, 2018-10-31 Work is constantly reshaped by technological progress. New ways of production are adopted, markets expand, and societies evolve. But some changes provoke more attention than others, in part due to the vast uncertainty involved in making predictions about the future. The 2019 World Development Report will study how the nature of work is changing as a result of advances in technology today. Technological progress disrupts existing systems. A new social contract is needed to smooth the transition and guard against rising inequality. Significant investments in human capital throughout a person’s lifecycle are vital to this effort. If workers are to stay competitive against machines they need to train or retool existing skills. A social protection system that includes a minimum basic level of protection for workers and citizens can complement new forms of employment. Improved private sector policies to encourage startup activity and competition can help countries compete in the digital age. Governments also need to ensure that firms pay their fair share of taxes, in part to fund this new social contract. The 2019 World Development Report presents an analysis of these issues based upon the available evidence.

accelerating access to critical therapies for als act: Clinical Trials in Neurology Roberto J. Guiloff, 2013-03-14 Clinical Trials in Neurology comprehensively tackles the methodology and design of clinical trials in neurological disease. A general section deals with the ethical aspects, drug development and regulatory requirements, basic trial designs and the statistics used. A diseases section tackles specific aspects of disorders, focusing on the relevant ethical issues, outcome variables and experience with large multicentre trials.

accelerating access to critical therapies for als act: Making Markets for Vaccines Owen Barder, Alice Albright, Michael Kremer, Ruth Levine, 2005 A legacy of our generation -- Ch. 1. We need to invest more in vaccines -- Ch. 2. Promoting private investment in vaccine development -- Ch. 3. A market not a prize -- Ch. 4. Design choices -- Ch. 5. $3 billion per disease -- Ch. 6. Meeting industry requirements -- Ch. 7. How sponsors can do it.

accelerating access to critical therapies for als act: *A Nationwide Framework for Surveillance of Cardiovascular and Chronic Lung Diseases* Institute of Medicine, Board on Population Health and Public Health Practice, Committee on a National Surveillance System for Cardiovascular and Select Chronic Diseases, 2011-08-26 Chronic diseases are common and costly, yet they are also among the most preventable health problems. Comprehensive and accurate disease surveillance systems are needed to implement successful efforts which will reduce the burden of chronic diseases on the U.S. population. A number of sources of surveillance data-including population surveys, cohort studies, disease registries, administrative health data, and vital statistics-contribute critical information about chronic disease. But no central surveillance system provides the information needed to analyze how chronic disease impacts the U.S. population, to identify public health priorities, or to track the progress of preventive efforts. A Nationwide Framework for Surveillance of Cardiovascular and Chronic Lung Diseases outlines a conceptual framework for building a national chronic disease surveillance system focused primarily on cardiovascular and chronic lung diseases. This system should be capable of providing data on

disparities in incidence and prevalence of the diseases by race, ethnicity, socioeconomic status, and geographic region, along with data on disease risk factors, clinical care delivery, and functional health outcomes. This coordinated surveillance system is needed to integrate and expand existing information across the multiple levels of decision making in order to generate actionable, timely knowledge for a range of stakeholders at the local, state or regional, and national levels. The recommendations presented in A Nationwide Framework for Surveillance of Cardiovascular and Chronic Lung Diseases focus on data collection, resource allocation, monitoring activities, and implementation. The report also recommends that systems evolve along with new knowledge about emerging risk factors, advancing technologies, and new understanding of the basis for disease. This report will inform decision-making among federal health agencies, especially the Department of Health and Human Services; public health and clinical practitioners; non-governmental organizations; and policy makers, among others.

accelerating access to critical therapies for als act: Night Noise Guidelines for Europe Charlotte Hurtley, 2009 The WHO Regional Office for Europe set up a working group of experts to provide scientific advice to the Member States for the development of future legislation and policy action in the area of assessment and control of night noise exposure. The working group reviewed available scientific evidence on the health effects of night noise, and derived health-based guideline values. In December 2006, the working group and stakeholders from industry, government and nongovernmental organizations reviewed and reached general agreement on the guideline values and key texts for the final document of the Night noise guidelines for Europe. Considering the scientific evidence on the thresholds of night noise exposure indicated by Lnight,outside [L suffix night,outside] as defined in the Environmental Noise Directive (2002/49/EC), an Lnight, outside of 40 dB should be the target of the night noise guideline (NNG) to protect the public, including the most vulnerable groups such as children, the chronically ill and the elderly. Lnight,outside value of 55 dB is recommended as an interim target for the countries where the NNG cannot be achieved in the short term for various reasons, and where policy-makers choose to adopt a stepwise approach. These guidelines are applicable to the Member States of the European Region, and may be considered as an extension to, as well as an update of, the previous WHO Guidelines for community noise (1999). [Ed.]

accelerating access to critical therapies for als act: The Impact of Parental Involvement, Parental Support and Family Education on Pupil Achievement and Adjustment Charles Desforges, 2003

accelerating access to critical therapies for als act: Equity, Social Determinants and Public Health Programmes World Health Organization, 2010 1. Introduction and methods of work.-- 2. Alcohol: equity and social determinants.-- 3. Cardiovascular disease: equity and social determinants.-- 4. Health and nutrition of children: equity and social determinants.-- 5. Diabetes: equity and social determinants.-- 6. Food safety: equity and social determinants.-- 7. Mental disorders: equity and social determinants.-- 8. Neglected tropical diseases: equity and social determinants.-- 9. Oral health: equity and social determinants.-- 10. Unintended pregnancy and pregnancy outcome: equity and social determinants.-- 11. Tobacco use: equity and social determinants.-- 12. Tuberculosis: the role of risk factors and social determinants.-- 13. Violence and unintentional injury: equity and social determinants.-- 14. Synergy for equity.

accelerating access to critical therapies for als act: Assisted Dying for the Terminally Ill Bill (HL) Great Britain: Parliament: House of Lords: Select Committee on the Assisted Dying for the Terminally Ill Bill, 2005-04-28 The Bill was published as HLB 4, session 2004-05 (ISBN 01084188390). This volume contains a selection of the 14,000 personal letters and other submissions received by the Committee with regards to their inquiry into the Bill.

accelerating access to critical therapies for als act: *The World Health Report 2003* World Health Organization, 2003 We have a real opportunity now to make progress that will mean longer healthier lives for millions of people.

accelerating access to critical therapies for als act: Health Literacy Who Regional Office for

Europe, 2013 As societies grow more complex and people are increasingly bombarded with health information and misinformation, health literacy becomes essential. People with strong health literacy skills enjoy better health and well-being, while those with weaker skills tend to engage in riskier behavior and have poorer health. With evidence from the recent European Health Literacy Survey, this report identifies practical and effective ways public health and other sector authorities and advocates can strengthen health literacy in a variety of settings, including educational settings, workplaces, marketplaces, health systems, new and traditional media and political arenas. The report can be used as a tool for spreading awareness, stimulating debate and research and, above all, for informing policy development and action.

accelerating access to critical therapies for als act: Global Tuberculosis Report 2019 World Health Organization, 2019-08-12 WHO has published a global TB report every year since 1997. The main aim of the report is to provide a comprehensive and up-to-date assessment of the TB epidemic, and of progress in prevention, diagnosis and treatment of the disease, at global, regional and country levels. This is done in the context of recommended global TB strategies and targets endorsed by WHO?s Member States, broader development goals set by the United Nations (UN) and targets set in the political declaration at the first UN high-level meeting on TB (held in September 2018). The 2019 edition of the global TB report was released on 17 October 2019. The data in this report are updated annually. Please note that direct comparisons between estimates of TB disease burden in the latest report and previous reports are not appropriate. The most recent time-series of estimates are published in the 2019 global TB report.

accelerating access to critical therapies for als act: Pharmaceuticals, Corporate Crime and Public Health Graham Dukes, John Braithwaite, J P Moloney, 2014-06-27 The pharmaceutical industry exists to serve the community, but over the years it has engaged massively in corporate crime, with the public footing the bill. This readable study by experts in medicine, law, criminology and public health documents the pr

accelerating access to critical therapies for als act: Health Policy and European Union Enlargement Mckee, 2004-04-01 While there may be consensus on the broader issues of the core objectives of the health care system, expectations differ between EU countries, and European national policy-makers. This book seeks firstly to assess the impact of the enlargement process and then to analyse the challenges that lie ahead in the field of health and health policy.

accelerating access to critical therapies for als act: *Toxicological Profile for Lead* , 2007

accelerating access to critical therapies for als act: Spinal Cord Injury Institute of Medicine, Board on Neuroscience and Behavioral Health, Committee on Spinal Cord Injury, 2005-08-27 An estimated 11,000 spinal cord injuries occur each year in the United States and more than 200,000 Americans suffer from maladies associated with spinal cord injury. This includes paralysis, bowel and bladder dysfunction, sexual dysfunction, respiratory impairment, temperature regulation problems, and chronic pain. During the last two decades, longstanding beliefs about the inability of the adult central nervous system to heal itself have been eroded by the flood of new information from research in the neurosciences and related fields. However, there are still no cures and the challenge of restoring function in the wake of spinal cord injuries remains extremely complex. Spinal Cord Injury examines the future directions for research with the goal to accelerate the development of cures for spinal cord injuries. While many of the recommendations are framed within the context of the specific needs articulated by the New York Spinal Cord Injury Research Board, the Institute of Medicine's panel of experts looked very broadly at research priorities relating to future directions for the field in general and make recommendations to strengthen and coordinate the existing infrastructure. Funders at federal and state agencies, academic organizations, pharmaceutical and device companies, and non-profit organizations will all find this book to be an essential resource as they examine their opportunities.

accelerating access to critical therapies for als act: Sharing Clinical Trial Data Institute of Medicine, Board on Health Sciences Policy, Committee on Strategies for Responsible Sharing of Clinical Trial Data, 2015-04-20 Data sharing can accelerate new discoveries by avoiding duplicative

trials, stimulating new ideas for research, and enabling the maximal scientific knowledge and benefits to be gained from the efforts of clinical trial participants and investigators. At the same time, sharing clinical trial data presents risks, burdens, and challenges. These include the need to protect the privacy and honor the consent of clinical trial participants; safeguard the legitimate economic interests of sponsors; and guard against invalid secondary analyses, which could undermine trust in clinical trials or otherwise harm public health. Sharing Clinical Trial Data presents activities and strategies for the responsible sharing of clinical trial data. With the goal of increasing scientific knowledge to lead to better therapies for patients, this book identifies guiding principles and makes recommendations to maximize the benefits and minimize risks. This report offers guidance on the types of clinical trial data available at different points in the process, the points in the process at which each type of data should be shared, methods for sharing data, what groups should have access to data, and future knowledge and infrastructure needs. Responsible sharing of clinical trial data will allow other investigators to replicate published findings and carry out additional analyses, strengthen the evidence base for regulatory and clinical decisions, and increase the scientific knowledge gained from investments by the funders of clinical trials. The recommendations of Sharing Clinical Trial Data will be useful both now and well into the future as improved sharing of data leads to a stronger evidence base for treatment. This book will be of interest to stakeholders across the spectrum of research-from funders, to researchers, to journals, to physicians, and ultimately, to patients.

accelerating access to critical therapies for als act: Global Monitoring Report 2014/2015 World Bank;International Monetary Fund, 2014-10-21 The Global Monitoring Report 2014/2015: Ending Poverty and Sharing Prosperity was written jointly by the World Bank Group (WBG) and the International Monetary Fund, with substantive inputs from the Organisation for Economic Co-operation and Development. This year's report details, for the first time, progress toward the WBG's twin goals of ending extreme poverty by 2030 and promoting shared prosperity and assesses the state of policies and institutions that are important for achieving them. The report continues to monitor progress on the Millennium Development Goals (MDGs). Also for the first time, the report includes information about high-income countries. It finds that while gaps in living standards have been closing in many countries, the well-being of households in the bottom 40 percent, as measured by the non-income MDGs such as access to education and health services, remains below that of households in the top 60 percent. The focus of this year's report is on three elements needed to make growth more inclusive and sustainable: investment in human capital that favors the poor, the best use of safety nets, and steps to ensure the environmental sustainability of economic growth. These three elements are imperative to all countries' development strategies, and are also fundamental to global efforts to achieve the twin goals, the MDGs, and the Sustainable Development Goals that will succeed the MDGs. Global Monitoring Report 2014/2015 was prepared in collaboration with regional development banks and other multilateral partners.

accelerating access to critical therapies for als act: *Dance Me to the End* Alison Acheson, 2019-10-08 A profoundly honest and intensely personal story of a woman who cares for her husband after the devastating terminal diagnosis of ALS. Marty, age 57, was given a preliminary diagnosis of ALS by his family doctor. Seven weeks later, the diagnosis was confirmed by a neurologist. Ten months and ten days later, Marty passed away. From day one, Alison, Marty’s spouse of over twenty-five years, kept a journal as a way to navigate the overwhelming state of her mind and soul. Soon the rawness of her words harmonized to tell the story of Marty’s diagnosis, illness, and decline. Her journal became a chronicle of caregiving as well as an emotional exploration of the tensions between the intuitive and the pragmatic, the logical and illogical, and the all-consuming demands of being both spouse and nurse. Divided into short pieces, some of which reads as free verse, Alison’s words are at times profoundly intense and painfully private. The composition of the intricate notes of a life in its final movements includes another stanza of the journal that became Dance Me to the End: the guiding of children grappling with the imminent loss of a parent, and the shifting roles of family, friends, and community—all of which add their own complex rhythms. Dance Me to the End

is an evocative memoir about the emotional impact of witnessing a loved one suffer from a neurological, degenerative, and terminal disease. This is a detailed account of grief, shock and pain coexisting with the levity, laughter and love shared with her husband and sons in those final months of Marty's life.

accelerating access to critical therapies for als act: *National EHealth Strategy Toolkit* World Health Organization, 2012 Worldwide the application of information and communication technologies to support national health-care services is rapidly expanding and increasingly important. This is especially so at a time when all health systems face stringent economic challenges and greater demands to provide more and better care especially to those most in need. The National eHealth Strategy Toolkit is an expert practical guide that provides governments their ministries and stakeholders with a solid foundation and method for the development and implementation of a national eHealth vision action plan and monitoring fram.

accelerating access to critical therapies for als act: Vital Directions for Health & Health Care Victor J. Dzau, Mark B. McClellan, J. Michael McGinnis, Elizabeth Finkelman, 2018-01-18 What can be more vital to each of us than our health? Yet, despite unprecedented health care spending, the U.S. health system is substantially underperforming, especially with respect to what should be possible, given current knowledge. Although the United States is currently devoting 18% of its Gross Domestic Product to delivering medical care¿more than $3 trillion annually and nearly double the expenditure of other advanced industrialized countries¿the U.S. health system ranked only 37th in performance in a World Health Organization assessment of member nations. In Vital Directions for Health & Health Care: An Initiative of the National Academy of Medicine, the U.S. National Academy of Medicine (NAM, formerly the Institute of Medicine), which has long stood as the nation¿s most trusted independent source of guidance in health, health care, and biomedical science, has marshaled the wisdom of more than 150 of the nation¿s best researchers and health policy experts to assess opportunities for substantially improving the health and well-being of Americans, the quality of care delivered, and the contributions of science and technology. This publication identifies practical and affordable steps that can and must be taken across eight action and infrastructure priorities, ranging from paying for value and connecting care, to measuring what matters most and accelerating the capture of real-world evidence. Without obscuring the difficulty of the changes needed, in Vital Directions, the NAM offers an important blueprint and resource for health, policy, and leaders at all levels to achieve much better health outcomes at much lower cost.

accelerating access to critical therapies for als act: Hold On, Let Go Nadine Sands, Michael Sands, 2016-10-12 This stay positive in difficult times story is about a man with ALS (also known as Lou Gehrig's disease) and his wife who writes about their inspiring journey in her blog called ALS With Courage. In Hold On, Let Go, Nadine Sands expands on her blog, which takes her readers from pre-diagnosis to Michael's complete paralysis, and reflects throughout on her husband's strength and determination; on their hope and faith in God; and the sorrow and joy of learning to let go. Mike has let go of working, walking, talking, eating, moving, and slowly he lets go of breathing. And I am letting go of him.... With each loss, we gain; by losing so much, we have more than we could imagine. Follow Nadine as she learns to savour every moment, to give thanks in all circumstances, and to cherish the love. Our love would never have known these depths had Mike been taken away suddenly. Instead we were given three to five years to rejoice, pray, and give thanks together every day.

accelerating access to critical therapies for als act: *Achieving Sustainable Development and Promoting Development Cooperation* Department of Economic & Social Affairs, United Nations, United Nations. Office for ECOSOC Support and Coordination, 2008 This book presents an overview of the key debates that took place during the Economic and Social Council meetings at the 2007 High-level Segment, at which ECOSOC organized its first biennial Development Cooperation Forum. The discussions also revolved around the theme of the second Annual Ministerial Review, Implementing the internationally agreed goals and commitments in regard to sustainable development.--P. 4 of cover.

accelerating access to critical therapies for als act: Augie's Quest Augie Nieto, T.R. Pearson, 2011-01-15 What happens to orphan diseases that aren't big enough profit centers for the pharmaceutical industry or get caught in the web of government funding and academic research? Augie Nieto found out in the spring of 2005: At forty-seven years old he was diagnosed with ALS. Devastated, Augie struggled with depression and attempted suicide. When he awoke from a coma, with friends and family surrounding him, his sense of faith and mission were renewed. Fast-forward one year: Augie and his wife, Lynne, were profiled as Real Heroes on the Today show. Their determination to beat the disease had already led to major overhauls in the way ALS research was conducted. In partnership with the Muscular Dystrophy Association, Augie had set up a foundation and lab to coordinate and oversee ALS research and testing across the globe. By centralizing operations and championing an entrepreneurial approach to medical research, Augie and his team accomplished remarkable results in less than two years. Part inspiration, part business innovation, Augie's Quest illustrates how one person can make a meaningful difference. Praise for Augie Nieto: George H. W. Bush, former U.S. president: Your contributions to the fitness industry are well known, and your dedication an inspiration. Lance Armstrong, seven-time Le Tour de France champion: I think it says a lot about Augie, the fact that everybody came together regardless of whether or not it's for competing gyms or competing companies that make equipment. They all say, this is one of our own; this is a guy who's committed his life to our industry and has been dealt a serious blow. We're going to be there for him, we're going to say, we're here for you and we're part of the Quest. Arnold Schwarzenegger, governor of California: Your success has been incredible. But you are so much more than just a successful businessman. You are the greatest husband, a great father, and a terrific friend. So,

accelerating access to critical therapies for als act: Making the Declaration Work Claire Charters, Rodolfo Stavenhagen, 2009 The United Nations Declaration on the Rights of Indigenous Peoples is a culmination of a centuries-long struggle by indigenous peoples for justice. It is an important new addition to UN human rights instruments in that it promotes equality for the world's indigenous peoples and recognizes their collective rights.--Back cover.

Additional Resources

ACCELERATING Definition & Meaning - Merriam-Webster
The meaning of ACCELERATING is increasing in speed or rate of occurrence. How to use accelerating in a sentence.

ACCELERATING | English meaning - Cambridge Dictionary
at an accelerating pace Since the crash, the value of the currency has been falling at an accelerating pace. at an accelerating rate Arctic Ocean ice is shrinking at an accelerating rate. ...

ACCELERATE Definition & Meaning - Merriam-Webster
The meaning of ACCELERATE is to move faster : to gain speed. How to use accelerate in a sentence.

Accelerating - definition of accelerating by The Free Dictionary
To increase the speed of: accelerated the engine. See Synonyms at speed. b. Physics To change the velocity of. 2. To cause to occur sooner than expected: accelerated his retirement by a ...

ACCELERATE Definition & Meaning - Dictionary.com
Accelerate definition: to cause faster or greater activity, development, progress, advancement, etc., in.. See examples of ACCELERATE used in a sentence.

Acceleration | Definition, Facts, & Units | Britannica

Jun 4, 2025 · acceleration, rate at which velocity changes with time, in terms of both speed and direction. A point or an object moving in a straight line is accelerated if it speeds up or slows ...

ACCELERATE | definition in the Cambridge English Dictionary
accelerate The vehicle accelerated around the turn. If a person or object accelerates, he, she, or it goes faster. Inflation is likely to accelerate this year, adding further upward pressure on ...

ACCELERATE definition and meaning | Collins English Dictionary
Growth will accelerate to 2.9% next year. [VERB] The government is to accelerate its privatisation programme. [VERB noun] When a moving vehicle accelerates, it goes faster and faster. ...

Accelerate - Definition, Meaning & Synonyms - Vocabulary.com
Accelerate means to speed up. A car accelerates when you step on the gas. You can accelerate the process of getting a visa if you happen to know someone who works in the consulate.

Acceleration Academies: Flexible and Supportive Approach to ...
Our unique model gives students the flexibility and support they need to earn their high school diploma. Discover a different high school experience today.

ACCELERATING Definition & Meaning - Merriam-Webster
The meaning of ACCELERATING is increasing in speed or rate of occurrence. How to use accelerating in a sentence.

ACCELERATING | English meaning - Cambridge Dictionary
at an accelerating pace Since the crash, the value of the currency has been falling at an accelerating pace. at an accelerating rate Arctic Ocean ice is shrinking at an accelerating rate. ...

ACCELERATE Definition & Meaning - Merriam-Webster
The meaning of ACCELERATE is to move faster : to gain speed. How to use accelerate in a sentence.

Accelerating - definition of accelerating by The Free Dictionary
To increase the speed of: accelerated the engine. See Synonyms at speed. b. Physics To change the velocity of. 2. To cause to occur sooner than expected: accelerated his retirement by a ...

ACCELERATE Definition & Meaning - Dictionary.com
Accelerate definition: to cause faster or greater activity, development, progress, advancement, etc., in.. See examples of ACCELERATE used in a sentence.

Acceleration | Definition, Facts, & Units | Britannica
Jun 4, 2025 · acceleration, rate at which velocity changes with time, in terms of both speed and direction. A point or an object moving in a straight line is accelerated if it speeds up or slows ...

ACCELERATE | definition in the Cambridge English Dictionary
accelerate The vehicle accelerated around the turn. If a person or object accelerates, he, she, or it goes faster. Inflation is likely to accelerate this year, adding further upward pressure on ...

ACCELERATE definition and meaning | Collins English Dictionary
Growth will accelerate to 2.9% next year. [VERB] The government is to accelerate its privatisation programme. [VERB noun] When a moving vehicle accelerates, it goes faster and faster. ...

Accelerate - Definition, Meaning & Synonyms - Vocabulary.com

Accelerate means to speed up. A car accelerates when you step on the gas. You can accelerate the process of getting a visa if you happen to know someone who works in the consulate.

Acceleration Academies: Flexible and Supportive Approach to ...
Our unique model gives students the flexibility and support they need to earn their high school diploma. Discover a different high school experience today.

Accelerating Access To Critical Therapies For Als Act

Accelerating Access To Critical Therapies For Als Act

Related Articles

- access control wiring diagram
- account management commission structure
- accounting aptitude test questions and answers pdf

Back to Home