

Comparative Effectiveness Research: The View from NIH

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Greetings from the National Institutes of Health



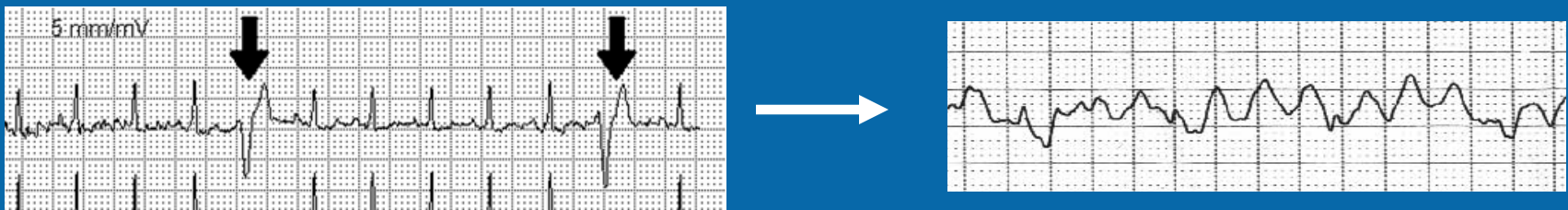
- Who we are
- What we do
 - 3 stories: sudden death, eye disease, cancer
 - Ongoing comparative effectiveness studies
 - Research infrastructure: CTSAs, Networks
- Thoughts about the future of CER

Who We Are

- Primary federal agency for conducting and supporting biomedical research
- 27 Institutes/Centers
- Over 18,000 employees
- Budget approximately \$30 billion
- Clinical research approximately \$9 billion

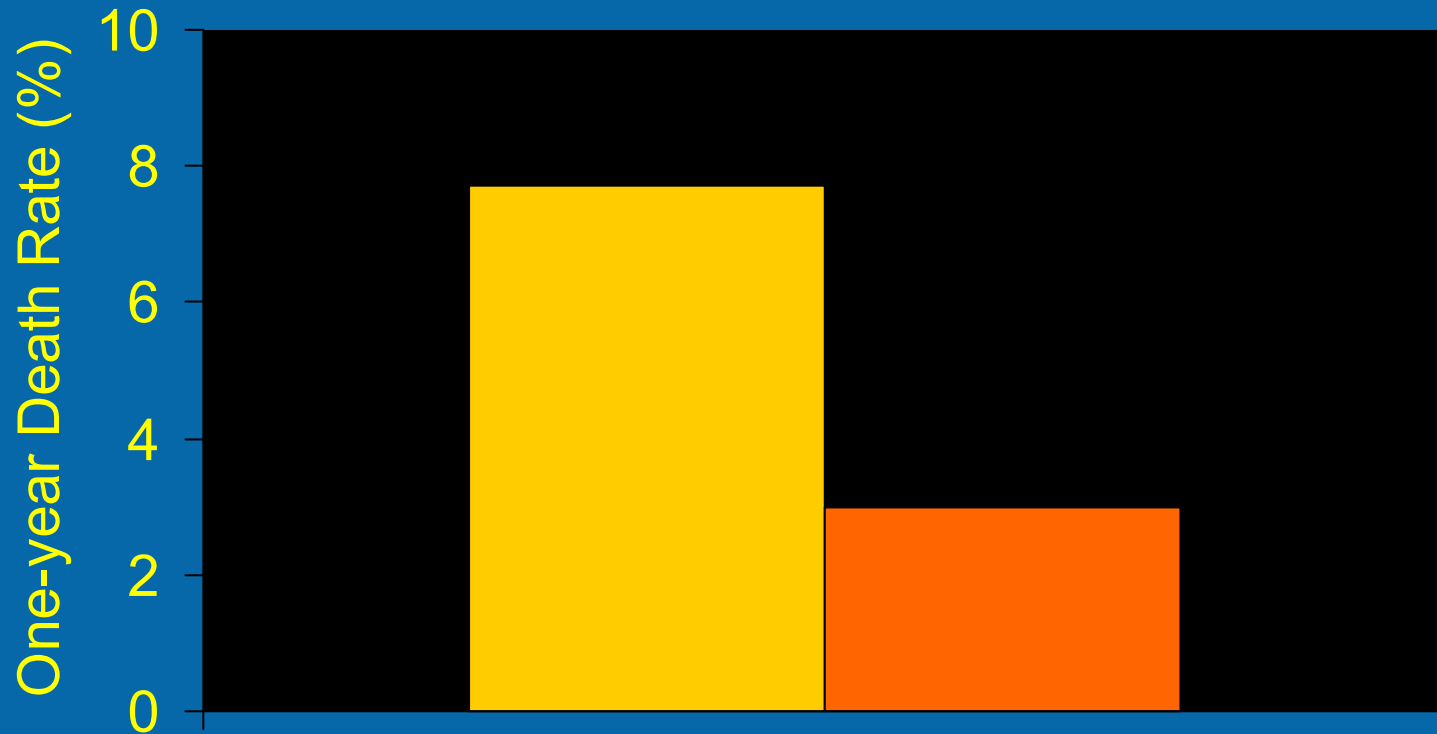
The First Story: Sudden Cardiac Death

- 1000 times a day in the US
- Major cause is “arrhythmia”
- 1980s: Abnormal beats predict death



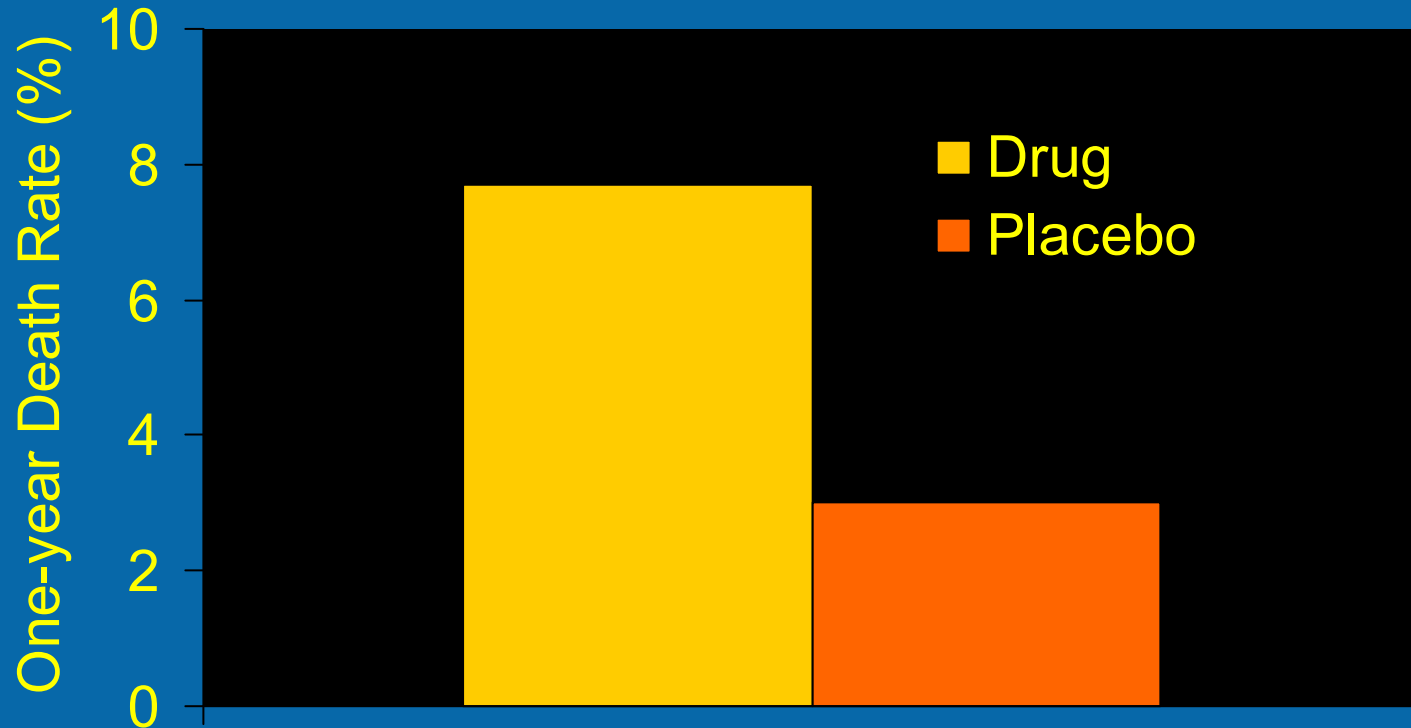
Logic: Get rid of abnormal beats, save lives

NIH Trial: Cardiac Arrhythmia Suppression



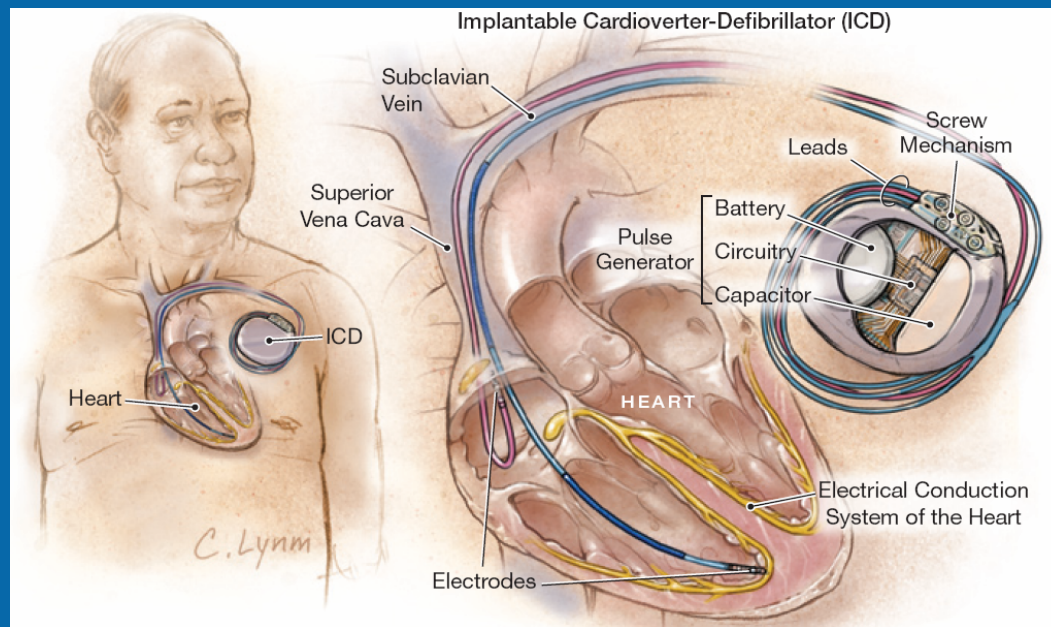
Approved drug given to 730; placebo to 725

NIH Trial: Cardiac Arrhythmia Suppression



Approved drug given to 730; placebo to 725

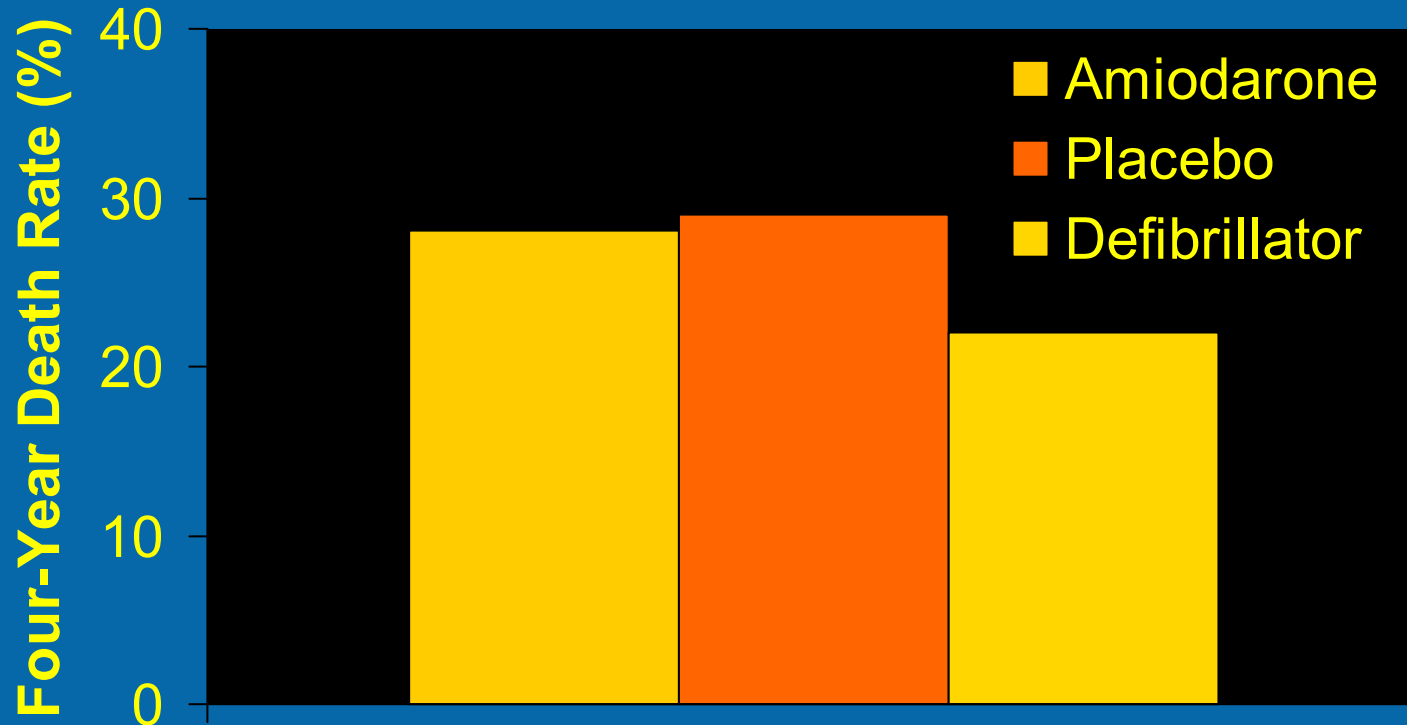
New Technology: Does it Work?



VS.



NIH Trial: Sudden Cardiac Death Heart Failure Trial



Amiodarone 845; placebo 847; defibrillator 829

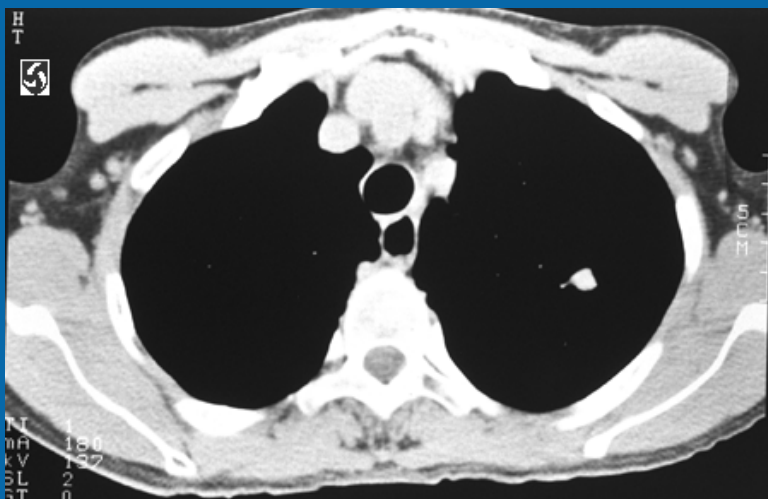
The Second Story: Macular Degeneration



- Lucentis: helps, but expensive
- Avastin: cancer approved, cheaper, less frequent injections
- CATT: Comparison of AMD Treatments

The Third Story: Lung Cancer Screening

- Major cause of death in smokers
- Can CT scans detect early, curable disease?
- NLST: National Lung Screening Trial



vs.



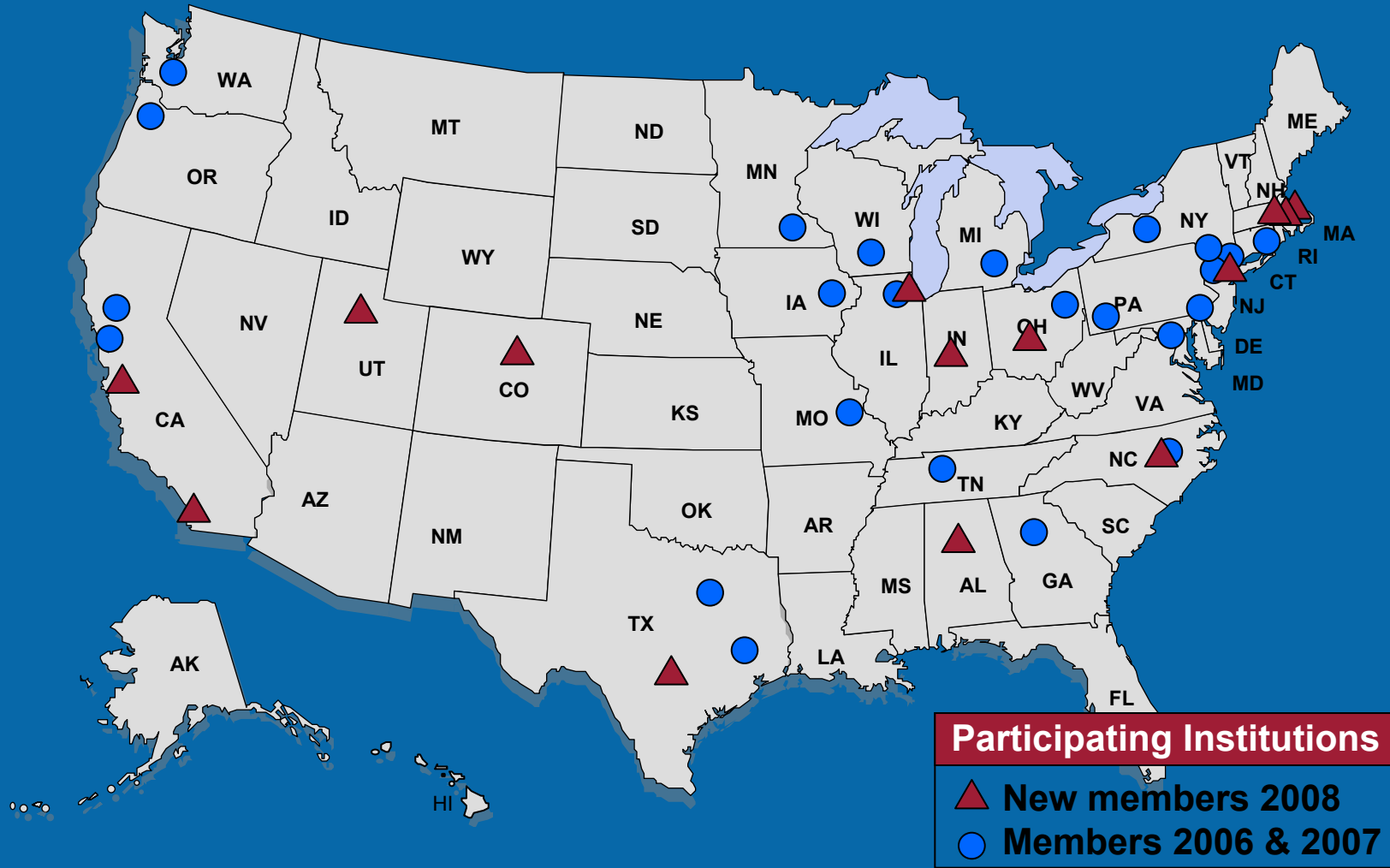
Comparative Effectiveness Research at NIH

- CBO Report defines CER as “...a rigorous evaluation of the impact of different options that are available for treating a given medical condition, for a particular set of patients.”
 - ...may compare similar treatments, such as competing drugs-- or analyze different approaches, such as surgery vs. drug
 - ...may focus only on the relative medical risks and benefits-- or may weigh both costs and benefits
- NIH supports at least \$627 million/year for Effectiveness Research and \$155 million/year for Cost Effectiveness Research

CER at NIH: A Long and Rich History

- Coronary artery surgery study (CASS)
- Antihypertensive and Lipid-lowering Treatment to Prevent Heart Attack (ALLHAT)
- National Emphysema Treatment Trial (NETT)
- Diabetes Prevention Program (DPP)
- Clinical Antipsychotic Trials of Intervention Effectiveness (CATIE)
- Action to Control Cardiovascular Risk in Diabetes Trial (ACCORD)
- Systolic Blood Pressure Intervention Trial (SPRINT)
- HMO Research Network (HMO RN)
- Colon cancer screening with CT
- Treatment of Early Onset Schizophrenia (TEOSS)

Clinical and Translational Science Awards (CTSA) Building a National Consortium of Academic Health Centers



Clinical Trials Research Networks

- AIDS malignancies
- Asthma
- Leukemia
- Drug-induced liver injury
- Bariatric surgery
- Maternal-fetal medicine
- Alzheimer's disease
- Drug abuse
-many, many more

National Library of Medicine CER Database

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1: [Maier O, De Jonge J, Nomden A, Hoekstra D, Baron W.](#)
Lovastatin induces the formation of abnormal myelin-like membrane sheets in primary oligodendrocytes.
Glia. 2008 Sep 23. [Epub ahead of print]
PMID: 18814266 [PubMed - as supplied by publisher]

2: [Sandhya VG, Rajamohan T.](#) [Related Articles](#)
Comparative evaluation of the hypolipidemic effects of coconut water and lovastatin in rats fed fat-cholesterol enriched diet.
Food Chem Toxicol. 2008 Sep 3. [Epub ahead of print]
PMID: 18809454 [PubMed - as supplied by publisher]

Lovastatin

- ▶ How do statins compare in reducing "bad cholesterol" (LDL-c)?
- ▶ In patients with diabetes or hyperlipidemia, how do combination drugs compare in improving long-term health?

Source: PubMed Clinical Q&A.

National Library of Medicine and CER

PubMed Clinical Q&A

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Summaries of key questions from the [Drug Effectiveness Review Project \(DERP\)](#). [Oregon Health & Science University](#)

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Comparing Statins

Laura Dean, MD

National Center of Biotechnology Information (NCBI)

Created December 1, 2007.

An important risk factor for coronary heart disease (CHD) is a raised cholesterol level, in particular, a high level of the "bad cholesterol" known as LDL-c. In patients who require drug therapy to lower their level of LDL-c, statins are the first-line agents.

The target LDL-c level depends on a patient's risk of heart disease, medical history, and initial cholesterol level. For most patients, the target will be <130mg/dL or <100mg/dL.

The "[Drug Class Review on HMG-CoA Reductase Inhibitors \(Statins\)](#)" compares the safety and effectiveness of [six statins](#). A summary of the findings is below.

How do statins compare in reducing "bad cholesterol" (LDL-c)?

All of the currently available statins are effective for reducing the level of LDL-c by up to 35%.

For patients who require their LDL-c level to be reduced by 35% to 50%, the following statins are effective:

- Atorvastatin 20 mg or more
- Lovastatin 80 mg

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- [How do statins compare increasing "good cholesterol" \(HDL-c\)?](#)
- [How do statins compare in decreasing mortality?](#)
- [Which statins reduce the risk of having a stroke or a heart attack?](#)
- [Does gender, age, or ethnicity influence the safety and effectiveness of statins?](#)
- [How do statins compare in people with diabetes?](#)
- [Are some statins more likely to cause muscle and liver damage?](#)
- [Drugs included in this review](#)

The Past and Present of CER at NIH

- Long, rich history
- Major ongoing trials and observational studies
- Breadth and depth of expertise
 - Physician scientists and clinical trialists
 - Statisticians
- Rigorous academic process relatively free from political pressure

NIH Does Prioritize CER: One IC's Strategic Plan



“To generate an improved understanding of the processes involved in translating research into practice...evaluate the risks, benefits, and costs of diagnostic tests and treatments in representative populations and settings...”

Thoughts about the Future of CER

- Role of trials versus observational studies
- Establishing priorities
- Optimally using existing resources
- Coordinating federal agencies and other research collaborators
- Improving research methods
- What's the importance of cost?