
Targeting Safe and Effective Prevention and Treatment: Industry Perspective

Simeon Taylor, M.D., Ph.D
Pharmaceutical Research Institute
Bristol-Myers Squibb Company

May, 2004

Objectives for Pharmaceutical R&D

- * Safe and effective drugs
- * Address unmet medical needs
- * To extend and enhance human life

Challenges for Pharmaceutical R&D

- * Cost-effective drug development
- * Increase success rates (especially, in late stages of process)
 - “Fail fast”
 - Obtain predictive information to make “go/no-go” decisions early in the process

Research and Development Process

Drug
Discovery



Research and Development Process

Drug Discovery



Clinical Development



Selected Opportunities for Public-Private Partnership

- * Basic scientific research
- * Translational research
- * Regulatory policy

Basic Research

- * **Novel target identification and validation**
 - Efficacy
 - Potential for mechanism-based toxicity
- * **Biomarkers**
 - Evidence for inhibiting target (dose-response)
 - Identify patients at risk to develop toxicity
 - Optimize dose in clinical practice

Translational Research

- * **Surrogate markers**

- Predict clinically relevant outcomes
- Accepted as basis for regulatory approval

- * **Large, longitudinal clinical studies**

- Diabetes Control and Complications Trial
- UK Prospective Diabetes Study
- Epidemiology of diabetes (Pima Indian studies)

HbA1c Predicts Prevention of Diabetic Complications

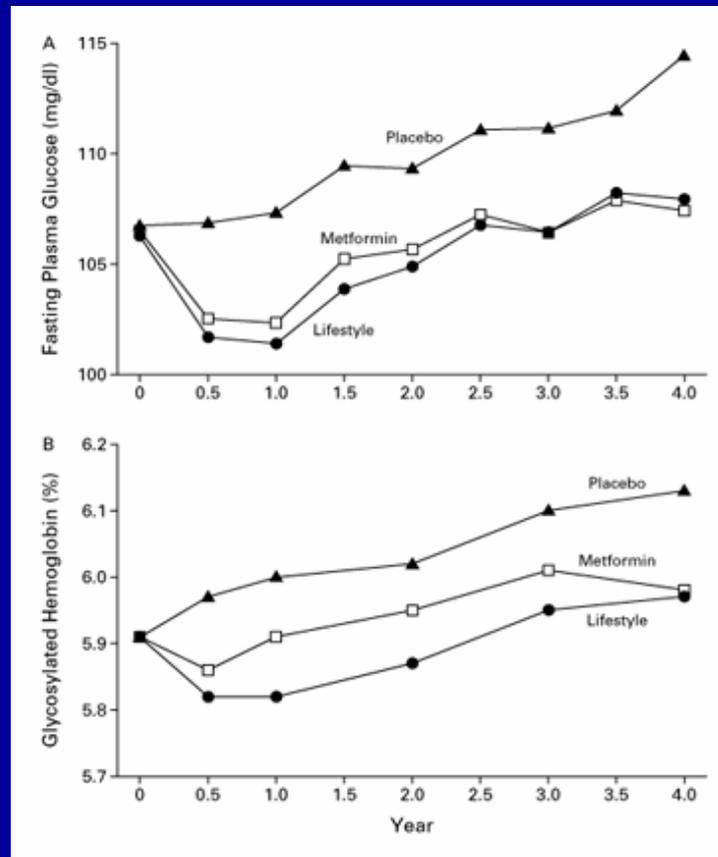
HbA1c Predicts Prevention of Diabetic Complications

The Diabetes Control and Complications Trial Research Group (NIDDK)

The Effect of Intensive Treatment of Diabetes on the
Development and Progression of Long-Term
Complications in Insulin-Dependent Diabetes Mellitus

New England Journal of Medicine, **329**: 977-986, 1993

Diabetes Prevention Program Research Group (NIDDK)



N Engl J Med, 346: 393-402, 2002

Targeting Unmet Medical Needs

- * **Selected examples**
 - Diabetes prevention
 - Metabolic syndrome
 - Chronic complications of diabetes
- * **Pharmaceutical development would be facilitated by availability of clear criteria for approval of drugs (cf., type 2 diabetes or obesity)**

Together we succeed...

- * **Government, academia, private sector**
- * **Shared objectives**
 - Safe and effective drugs
 - Address unmet medical needs
 - To extend and enhance human life
- * **The patient wins!**