



Study Design & Quality Measures

Real World Evidence Demonstrates Real Results

Valeritas has focused on providing data that demonstrates clinical and economic benefits in a real-world setting

- Based on standard clinical practice
- Inclusive of a wide range of patients (good control to poor control)
- No forced insulin titration algorithms
- No mandated office visits or regular phone contact
- Patients pay for product and office visits copays

Randomized Controlled Trials vs Real World Evidence

Randomized Controlled Trials

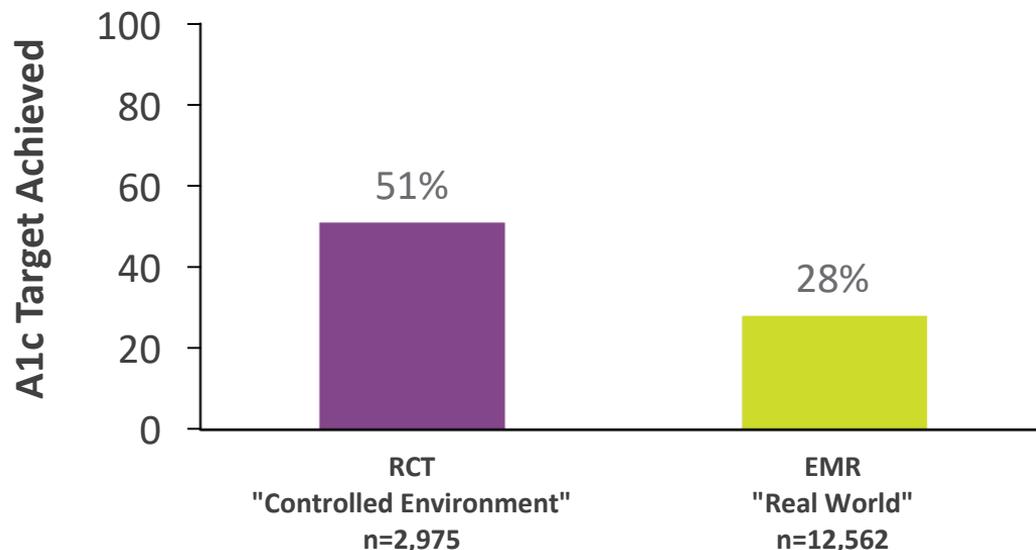
- Prospective in design
- Required for FDA submissions of pharmaceuticals, not devices
- Tests a pre-conceived hypothesis that may or may not be based on previous scientific observations
- Strict criteria for subjects studied; “sterile environment”
- Controls for concomitant diseases and medications; not “real-world”
- Tests for impact of a single treatment modality

Real World Evidence

- Prospective or retrospective in design
- Real-world; few controls other than standards for comparison for scientific credibility
- Rich database already exists via clinical observations and assessment notes and can accessed
- Can compare several different treatment modalities in the same treatment environment
- Generates hypotheses for future prospective efficacy and safety studies

Disparity May Exist between Randomized Controlled Trials (RCTs) and Real World Clinical Practice

- RCTs follow restrictive/controlled methodologies and patients are carefully screened based on precise clinical criteria.
- Findings may not be generalizable to everyday clinical practice.



Study in patients prescribed basal insulin to evaluate achievement of A1C target (<7.0%) after 6 months across 11 pooled RCTs and 1 electronic medical record (EMR) database representing "real world" clinical practice

A Changing Healthcare Landscape

Quality Measures and Performance Standards

- Health plans and physicians are being called on to close gaps in care and improve overall quality
 - “Quality of Diabetes Care” is measured across all performance standards
- The Centers for Medicare and Medicaid (CMS) use quality measures and performance to
 - Allocate shared savings for ACO’s
 - Assign *Star Ratings* highlighting the quality of medical/health care services provided by a plan which can influence rebates/bonuses to plans and allow for comparisons between plans by beneficiaries.
- Commercial plans also use quality measures for accreditation and have financial incentives for improving performance based on scores