LapidusData Capabilities

David Lapidus
President, LapidusData Inc.
David@LapidusData.com
617-308-3975

About LapidusData Inc.

- Serving orphan drug developers since 2008:

- Expertise: Quantifying rare disease populations for commercial purposes

- Core activity: Market analytics for licensing, M&A, and commercialization

- Experience: Epidemiology analyses and forecasts for 60+ orphan drug companies and 130+ rare diseases

- David Lapidus, President

  – Previous roles: Market forecasting at Genzyme and Decision Resources

  – Speaker at World Orphan Drug Conference (Washington, DC), 2012-2022

Publications

Colburn R, Lapidus D. **An analysis of Pompe newborn screening data: a new prevalence at birth, insight and discussion**. Front Pediatr. 2024 Jan 8;11:1221140. doi: 10.3389/fped.2023.1221140. PMID: 38274468; PMCID: PMC10810242.

Lapidus D. **Strengths and limitations of new artificial intelligence tool for rare disease epidemiology**. J Transl Med. 2023 Apr 30;21(1):292. doi: 10.1186/s12967-023-04152-0. PMID: 37122037; PMCID: PMC10149020.

Howe SJ, Lapidus D, Hull M, Yeaw J, Stevenson T, Sampson JB. **Healthcare resource utilization, total costs, and comorbidities among patients with myotonic dystrophy using U.S. insurance claims data from 2012 to 2019**. Orphanet J Rare Dis. 2022 Feb 23;17(1):79. doi: 10.1186/s13023-022-02241-9. Erratum in: Orphanet J Rare Dis. 2022 Jul 11;17(1):260. PMID: 35197080; PMCID: PMC8867662.

Pignolo RJ, Hsiao EC, Baujat G, Lapidus D, Sherman A, Kaplan FS. **Prevalence of fibrodysplasia ossificans progressiva (FOP) in the United States: estimate from three treatment centers and a patient organization**. Orphanet J Rare Dis. 2021 Aug 5;16(1):350. doi: 10.1186/s13023-021-01983-2. PMID: 34353327; PMCID: PMC8340531.

Chiang A, Solis DC, Rogers H, Sohn GK, Cho HG, Saldanha G, Lapidus D, Li S, Sarin KY, Tang JY. **Prevalence and risk factors for high frequency basal cell carcinoma in the United States**. J Am Acad Dermatol. 2020 Jul 28:S0190-9622(20)32289-1.

Sellos-Moura M, Glavin F, Lapidus D, Evans KA, Palmer L, Irwin DE. **Estimated prevalence of moderate to severely elevated total homocysteine levels in the United States: A missed opportunity for diagnosis of homocystinuria?** Mol Genet Metab. 2020 May;130(1):36-40. doi: 10.1016/j.ymgme.2020.02.001. Epub 2020 Feb 5. PMID: 32057642.

Sellos-Moura M, Glavin F, Lapidus D, Evans K, Lew CR, Irwin DE. **Prevalence, characteristics, and costs of diagnosed homocystinuria, elevated homocysteine, and phenylketonuria in the United States: a retrospective claims-based comparison**. BMC Health Serv Res. 2020 Mar 6;20(1):183. doi: 10.1186/s12913-020-5054-5. PMID: 32143624; PMCID: PMC7059682.

Sellos-Moura M, Glavin JF, Horn PT, Lapidus D, Kraus J. **Claims-based analysis of homocysteine testing, elevated homocysteine levels, and homocystinuria diagnosis in the United States**. Value in Health. 2018 Oct; volume 21, S440 - S441.

Sellos-Moura M, Glavin JF, Horn PT, Lapidus D, Kraus J. **Comparison of the estimated prevalence of diagnosed homocystinuria and phenylketonuria in the United States.** Value in Health. 2018 Oct; volume 21, S441.

Baujat G, Choquet R, Bouée S, Jeanbat V, Courouve L, Ruel A, Michot C, Le Quan Sang KH, Lapidus D, Messiaen C, Landais P, Cormier-Daire V. **Prevalence of fibrodysplasia ossificans progressiva (FOP) in France: an estimate based on a record linkage of two national databases**. Orphanet J Rare Dis. 2017 Jun 30;12(1):123. doi: 10.1186/s13023-017-0674-5. PMID: 28666455; PMCID: PMC5493013.

Therrell BL, Currier R, Lapidus D, Grimm M, Cederbaum SD. **Newborn screening for hyperargininemia due to arginase 1 deficiency**. Mol Genet Metab. 2017 Aug;121(4):308-313. PubMed PMID: 28659245.

Cadieux B, Lapidus D, Greenbaum LA. **Adherence to cysteamine and kidney outcomes among 224 cystinosis patients.** Annual Meeting of the American Society of Pediatric Nephrology, Vancouver. 2014.

Rare Collective ™

- Trusted advisors in orphan drug development
  - Communications
  - Market Research
  - Market Sizing & Modeling
  - Patient Advocacy
  - Pricing & Reimbursement

- Experts in advancing rare disease therapies
  - Hands-on experience in all stages of development, commercialization
  - Across geographies – Intl capabilities w native speakers

- Proven ability to deliver results
  - Working across disciplines
  - Addressing specific needs

Left to Right
Doug Paul; MME
Patti Engel; Engage Health
Chris Smith; SmithSolve
Jean Campbell; J Campbell & Assoc.
David Lapidus; LapidusData

Introduction to LapidusData

What does LapidusData do?

Use "commercial epidemiology" to quantify patients with rare diseases

How does LapidusData do it?

Review & synthesize literature

Analyze large data sets (insurance claims, European administrative records)

Physician surveys

Other primary research

Why perform these activities?

Population models & revenue forecasts:
- Opportunity assessment
- Valuation
- Commercial planning
- Regulatory (ODD/PRV)

Why work with LapidusData?

- 15+ years of focus on rare diseases
- Statistical rigor
- Transparent forecast assumptions
- Defensibility through identification of forecast risk and sensitivity analysis
- Long-term view of analytic needs

Methods

Published literature

- Rigorous analysis of all available publications
- Creative use of connections among sparse data points to reach commercially-relevant conclusions
- Commitment to find/analyze original sources
- Leverage/add measures of statistical significance

Administrative data (insurance claims)

- Experience using large data sets for rare disease analyses (sample size, coding)
- Project management: ensure vendors provide reports that are commercially actionable
- Store/analyze data for exploratory and ad hoc queries (IT resources?)
- Uses: Prevalent, Dx, Tx, nat'l history, MD lists

Primary research collaborations (examples)

- Fibrodysplasia ossificans progressiva: Work with KOLS at European reference centers to extract national data on prevalence and natural history

- Urea cycle disorder: Gather data from states' newborn screening programs to estimate minimum incidence

- Rare bone disease: Survey physicians to identify treaters, referral patterns (specialties and local experts), diagnostics, etc.

Unique Approach to Forecast Models

Dynamic inputs, flexible reporting, integrated scenarios/sensitivity to understand risk

Granular segmentation

- ~80,000 forecast points in a *small* model
- Countries/regions
- Age groups
- Disease subtypes/severities
- Time periods: Quarters or months

Sensitivity analysis

- Low/med/high for all inputs
- ~20 input categories
- ~10 billion individual scenarios
- Simple "switchboard" to manage combinations

Integrated financial view

- Mkt penetration vs. prevalence & diagnosis
- Country-specific launch dates, uptake, and EAP
- Competitors and generics
- Country prices, annual increase/decrease
- Gross-to-net
- FTEs and expenses as individual line items

Interdependencies for commercial planning

- Changes to inputs are designed to cascade through forecast with minimum manual effort
- Market dynamics can automatically shift with launch dates: uptake, diagnosis, price changes, expense accruals, etc.
- Reference pricing: global linkages for pricing assumptions