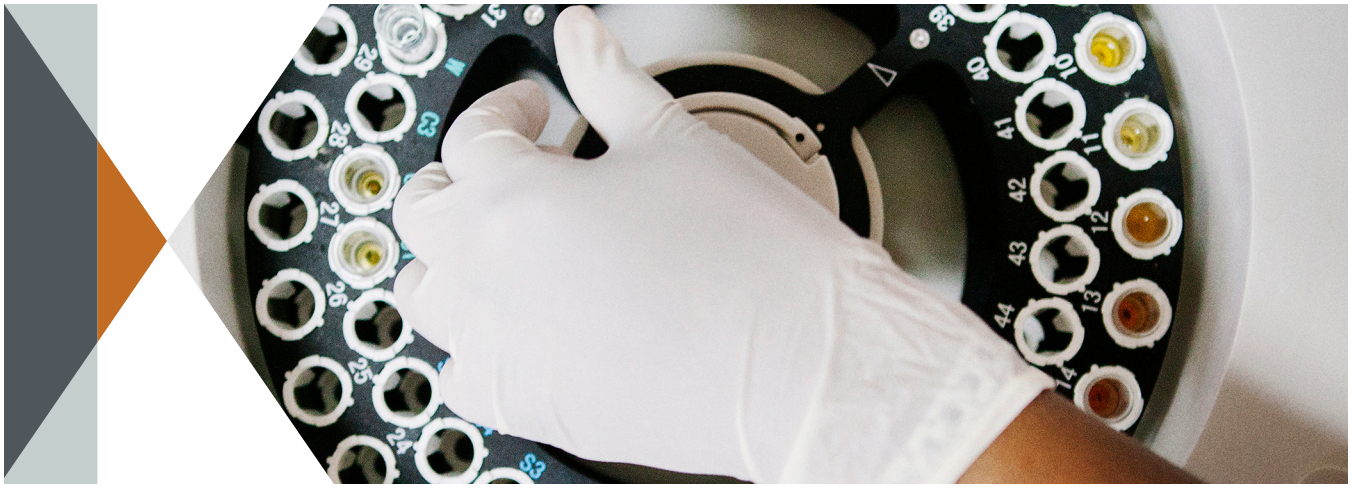


EXECUTIVE INSIGHTS

Looking Ahead in Biopharma: Key Trends Impacting the Industry

The year 2021 brought tremendous progress to the biopharma industry. Nearly 5 billion people have now been at least partially vaccinated against COVID-19 worldwide,[1] the first ever in-human data showing safety and efficacy of in vivo CRISPR gene editing was published,[2] and 128 therapeutic-focused biopharma companies went public globally.[3]

Going forward, biopharma executives will face challenges and should embrace transformation across many dimensions, including commercialization, financing and portfolio planning. This report shares highlights from five trends that are having the greatest impact on the industry:

1. Growth of the advanced modality pipeline
2. Continued global pricing pressure
3. Evolving commercialization models
4. Increased patient diagnosis and biomarker initiatives
5. Evolving financing landscape

L.E.K. Consulting has developed its analysis based on global events and developments currently impacting the biopharma landscape and the key trends to watch:

1. Growth of the advanced modality pipeline

The advanced modality pipeline has grown rapidly in the past five years, and 2021 marked another "year of firsts" for advanced modalities. China approved its first CAR-T therapy, the first BCMA-targeted CAR-T therapy was approved in the U.S.,[4] and Intellia announced the first positive human data for CRISPR in vivo gene editing. A record $23.1 billion was raised in

the areas of gene therapy, cell immunotherapy/cell therapy and tissue engineering.[5] Clinical, regulatory and commercial milestones in 2022 will shape the trajectory of an increasingly diverse advanced modality pipeline.

Key developments to watch include the following:

- Gilead's Yescarta and Bristol Myers Squibb's (BMS) Breyanzi are racing to **bring CAR-T to earlier lines of large B-cell lymphoma treatment**. Gilead filed regulatory submissions for second-line use with both the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA) and other regulatory authorities,[6] and BMS filed a submission for second-line use with the FDA.[7] Approvals would expand the market potential of these therapies.
- There are only two approved gene therapies in the U.S., but **multiple times that number may reach regulatory milestones this year**. For example, Bluebird is awaiting FDA review of biologics license applications for beti-cel gene therapy for transfusion-dependent ß-thalassemia[8] and eli-cel for cerebral adrenoleukodystrophy (CALD),[9] and BioMarin's valoctocogene roxaparvovec for severe hemophilia A is being reviewed by the EMA and may soon be submitted to the FDA.[10] Other planned or potential FDA and/or EMA submissions include CRISPR and Vertex's CTX001 for transfusion-dependent ß-thalassemia and severe sickle cell disease,[11] and CSL Behring and uniQure's etranacogene dezaparvovec for severe to moderately severe hemophilia B.[12] The decisions will set precedents for the safety and efficacy needed to support approval. The therapies' next steps in gaining market access, manufacturing at commercial scale and driving adoption will also be instructive for the field.
- The **reinvigoration of the RNA therapeutic pipeline** will likely continue in 2022. Expected late-stage readouts include Alnylam's RNAi Onpattro in transthyretin amyloidosis with cardiomyopathy (ATTR-CM)[13] and Ionis and AstraZeneca's antisense oligonucleotide eplontersen for hereditary ATTR polyneuropathy (hATTR-PN).[14] In addition, the pipeline could expand its focus in mechanisms like RNA editing, tRNA, circular RNA and mRNA.[15,16]
- Biopharmas are increasingly **looking at protein degradation** to destroy disease-causing proteins previously believed to be too difficult to pharmacologically target by using small molecules such as proteolysis-targeting chimeras (PROTACs).[17] The preclinical- and clinical-stage protein degrader pipeline increased by nearly 70% from 2020 to 2021,[18] and 2022 should be a year of continued progress.

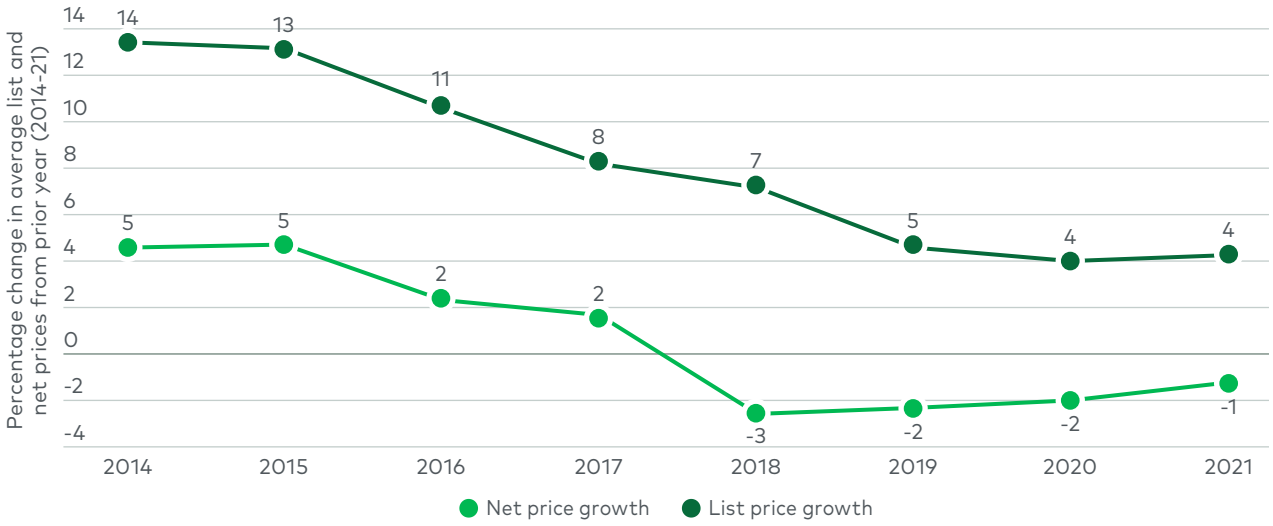
The developments listed above are by no means comprehensive. For example, we also expect to see continued investment in allogeneic cell therapy and rapid growth in the Chinese cell

and gene therapy pipeline. But they demonstrate clear advancements — from exploring new mechanisms to expanding to new diseases to accelerating in new geographies and setting critical precedents in the path to commercialization.

2. Continued global pricing pressure

Governments, payers and consumers continue to place pressure on drug prices across the globe. In the U.S., pharmacy benefit managers (PBMs) and payers are negotiating more significant discounts, with declining net prices since 2018 despite list price increases[19] (see Figure 1). Payers also are continuing to restrict coverage of therapies they deem too expensive; the Centers for Medicare & Medicaid Services' (CMS) recent proposed coverage restriction of Aduhelm to clinical trial patients[20] is just one example. Pricing pressure may also come from new competitors within the industry. EQRx, the first of a potential wave of companies with disruptive "low-cost, me-too" strategies, intends to price its therapies 50%-70% lower than competitors' pricing.[21] It has assembled a pipeline of at least five clinical assets[22] and has partnerships or memoranda of understanding with CVS Health, Geisinger, Blue Shield of California, BlueCross BlueShield of North Carolina, Horizon and NHS England.[23] The timing of its first filings remains to be seen, as their current pivotal trial data for two lead assets is from Chinese studies and U.S.-based studies planned to begin

Figure 1
Year-to-year list and net price growth for US branded drugs



Note: From Drug Channels Institute 2021 and 2022 reports: "Drug Channels Institute analysis of SSR Health data. List and estimated net pricing figures are based on data for approximately 1,000 brand-name drugs with disclosed U.S. product-level sales from approximately 100 currently or previously publicly traded firms. The products and companies account for more than 90% of U.S. branded prescription net sales. Net price equals list price minus off-invoice rebates and such other reductions as distribution fees, product returns, chargeback discounts to hospitals, price reductions from the 340B Drug Pricing Program, and other purchase discounts. List price data for 2021 reflects first three quarters only. Net price data for 2021 reflects four-quarter moving average." L.E.K. rounded to nearest percentage point.
Source: Drug Channels Institute analysis of SSR Health data; L.E.K. research and analysis

this year.[24] Additionally, the biosimilar pipeline is accelerating and may soon provide increased competition to numerous blockbuster franchises.

The Biden administration has set forth numerous proposed policies to control drug prices, such as Medicare drug price negotiation, penalties for price increases over inflation rates, earlier generic and biosimilar competition, and rebate transparency.[25,26] Some of these provisions are included in the latest Build Back Better bill,[27] though congressional gridlock makes it unclear whether and in what form they could be approved. Despite this, bipartisan pressure still exists for drug pricing reform, and smaller-scale changes in some form still could be imminent.

Beyond the U.S., countries across the globe also introduced new measures or plans to control pricing in 2021. For example, Germany's new government introduced a plan to reduce the free pricing period from 12 to six months, strengthen the authority of public insurers to limit drug prices, and extend price freezes beyond 2022.[28] Japan took its first "off-year" drug price cut and started using a cost-effectiveness assessment for expensive, innovative drugs.[29] China's National Healthcare Security Administration negotiated an average 61.7% discount for drugs newly added to the National Reimbursement Drug List — the most significant price cuts since 2017.[30]

Going forward, biopharmas will need to carefully balance the pricing-market access equation, and alternative pricing models like value-based/pay-for-performance contracts will be an important tool for doing so.

3. Evolving commercialization models

A confluence of forces, including the COVID-19 pandemic, hospital system consolidation, increased institutional decision-making and new advanced treatment modalities, is causing biopharmas to evolve their commercial models. We expect launches to further develop in the following ways:

- **Smaller sales forces with a mix of in-person and virtual engagement.** The traditional sales rep-based commercial model is giving way to a nimbler approach with fewer reps and more virtual touchpoints. The pandemic accelerated this shift, but it is here to stay: 66% of physicians in a May 2021 GlobalData survey expected a mix of virtual and in-person interactions post-pandemic.[31] Pharma companies have acted accordingly. Amgen cut 500 jobs last year, mainly from its U.S. sales force,[32] and this January Pfizer followed suit with sales staff reductions of an undisclosed size.[33] This shift will require reimagining the sales rep role and investing in significant retraining to deliver a satisfactory customer experience.

Artificial intelligence and machine learning will increasingly underlie this approach by detecting shifts in market dynamics that impact commercial deployment decisions well before a classic model of in-the-field reports would do so. Success will become more nuanced, requiring clear and compelling content shared through the right channels at the right times, and accounting for physicians' preferences, while maintaining a personal touch.

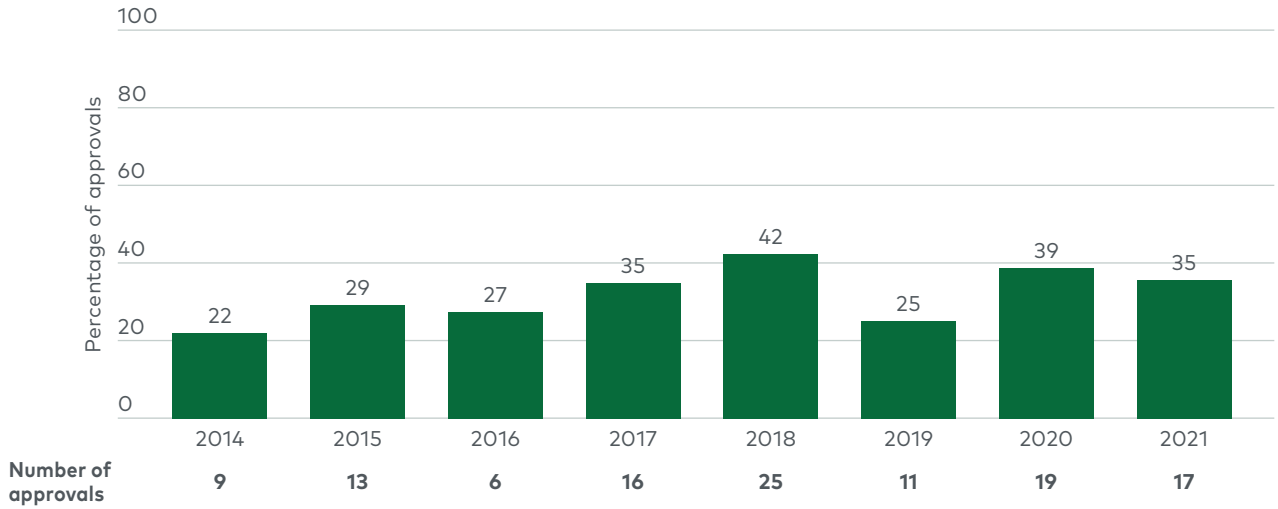
- **Greater focus on healthcare systems versus individual decision-makers.** Fundamental shifts in the definitions of "customer" and "decision-maker" are causing biopharmas to reexamine their commercial models. In the U.S., for example, hospital systems continue to consolidate, and an increasing proportion of physicians are employees rather than business owners.[34] As a result, launch teams are increasingly targeting institutional decision-makers at large healthcare systems. Novartis' launch strategy for its PCSK9 inhibitor, Leqvio, is a prime example. Instead of focusing on individual physicians as decision-makers, Novartis is hoping to work with approximately 200 hospital systems in the U.S. to help identify patients suitable for the therapy.[35] Novartis also has a population-level agreement with England's National Health Service (signed by The Medicines Co. before its acquisition by Novartis) to make the drug available for high-risk patients.[36] This more coordinated, centralized account planning strategy should become increasingly common going forward.
- **Increased mobilization of authorized treatment centers for advanced therapies.** Cell and gene therapies have necessitated a fundamentally different commercial model from that of traditional small-molecule therapies and biologics. Early entrants like Novartis, Gilead and Spark have set up networks of authorized treatment centers[37] to control quality, manage adverse events and oversee bidirectional manufacturing logistics where relevant. Success in this model requires site enrollment, activation, ongoing support and strong medical science liaisons, but relies much less on the traditional broad-based sales strategy. The cell and gene therapy space will likely come of age during this decade — there may be 60-plus product-indication approvals of durable[38] cell and gene therapies in the U.S. by 2030, for example[39] — and companies will need to mobilize very differently from how they are used to doing so.

4. Increased patient diagnosis and biomarker initiatives

Biomarker strategies for screening, diagnosis, treatment selection and response monitoring will become increasingly critical components of drug launches. Personalized medicines have accounted for at least one-third of the FDA's Center for Drug Evaluation and Research (CDER) therapeutic new molecular entity (NME) approvals for four of the past five years, compared with only 5% in 2005 (see Figure 2).[40]

Figure 2

Percentage and number of FDA therapeutic NME* CDER** approvals that were personalized medicines***



*New drugs, agents or therapeutic biologics
**Excludes Center for Biologics Evaluation and Research approvals such as cell and gene therapies
***From Personalized Medicine Coalition (PMC): "Methodology: When evaluating new molecular entities, PMC categorizes personalized medicines as those therapeutic products for which the label includes reference to specific biological markers, identified by diagnostic tools, that help guide decisions and/or procedures for their use in individual patients." L.E.K. rounded to nearest percentage point.
Source: Personalized Medicine Coalition's annual "Personalized Medicine at FDA" progress reports

In oncology, biomarker involvement in the patient journey is becoming more intricate and nuanced:

- Advances in liquid biopsy are beginning to enable more **widely available screening and earlier diagnosis**. For example, Exact Sciences[41] and Grail[42] are developing or have developed blood-based multicancer early detection tests. More widespread availability of tests like this could allow biopharmas to increase patient flow into the treatment paradigm.
- Liquid biopsy advances are also enabling better **response monitoring** in the adjuvant setting, which could drive new treatment protocols. For example, CMS is now covering Natera's Signatera ctDNA MRD monitoring in stage II-III colorectal cancer for use in the post-surgical setting to detect possible recurrence at an average of 8.7 months earlier than existing diagnostics can.[43]
- For **treatment selection**, tumor types are being segmented into smaller subtypes based on biomarkers. NSCLC is the prime example, but this is also occurring in breast, prostate and colorectal cancers. The pipeline for tumor subtype therapies will likely continue to expand. As patient populations for these therapies become smaller, higher prices may be required to support revenue potential.

The use of biomarkers is also poised to expand into applications beyond oncology, particularly in neurologic, immunologic and cardiovascular diseases. For example, there is a need for affordable, noninvasive tests to diagnose Alzheimer's disease and nonalcoholic steatohepatitis in very early, even pre-symptomatic, stages. Diagnostics companies are working to develop such tests, and biopharmas are beginning to incorporate them into their clinical trials. For example, C_2N Diagnostics' blood-based PrecivityAD test for beta-amyloid is being used for enrollment in a trial of Eisai's lecanemab, an anti-amyloid monoclonal antibody, in preclinical Alzheimer's disease.[44]

Key success factors for biopharma launches will increasingly include forming partnerships with diagnostics companies, ensuring test reimbursement, promoting test availability and educating physicians and patients. From the translational medicine stage, product planning teams must default to explicitly incorporating diagnostic strategies into their development and launch plans — and must opt out of this only if there is an inexpensive, easy-to-administer, validated and widely adopted biomarker for their target patient population.

5. Evolving financing landscape

The consensus in Western markets appears to be that public financing will decrease in favor of private and alternative fundraising. After strong volumes of global biotech IPOs in 2020 and 2021, IPOs are predicted to decelerate in 2022.[45] About 80% of 2021 biotech IPOs in the U.S. were trading below their offering prices at the end of the year, and the Nasdaq Biotechnology Index generated no return in 2021, versus >25% for the S&P 500.[46] Many special purpose acquisition companies (SPACs) have underperformed, and the U.S. Securities and Exchange Commission is likely to introduce new regulations that would eliminate some of SPACs' advantages.[47] With U.S. equity markets challenged, we expect continued strong venture capital investments and an increase in alternative, nondilutive financing options like royalty monetization and debt financing.

The HKEX in Hong Kong has become the second-largest IPO exchange for biotech.[48] It had a strong ramp-up since introducing a new listing regime in 2018, but then underwent a correction in the second half of 2021 that could impact public fundraising in 2022. There will likely be continued momentum in Asian venture capital markets, but alternative fundraising mechanisms are expected to remain uncommon.

Conclusion

Combined, these trends are leading to fundamental shifts across the healthcare ecosystem. To win in this evolving landscape, industry leaders will have to monitor these trends and adapt their corporate strategies to make decisions regarding portfolio investments, new

product launch strategies, capital formation and partnerships with industry participants. L.E.K. Consulting is closely monitoring developments across all these trends and can help biopharmas effectively respond to each of these strategic imperatives.

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