Biopharmaceutical Company Communications with Health Care Stakeholders

RESULTS OF SURVEYS WITH PAYERS AND PROVIDERS

PhRMA
RESEARCH • PROGRESS • HOPE

HealthStrategiesGroup®

DECEMBER 2017

Executive Summary

As biomedical research continues to push into new frontiers of medicine – targeting increasingly complex and challenging diseases – the private market is evolving alongside the science to ensure that patients and health care stakeholders are able to incorporate the best available evidence into their decision-making. In this new era of medicine, payers, providers and other stakeholders in the health care system are increasingly turning a critical eye toward evaluating innovative medicines to ensure that they provide added or differentiated benefits compared to existing products.

As innovative biopharmaceutical companies face increased demands for demonstrating value, and an increasingly dynamic scientific and clinical landscape, they also face barriers to communicating new research findings under the Federal Drug Administration's (FDA) regulatory paradigm. FDA has recognized this and begun taking steps to facilitate appropriate communication. However, additional legislative or administrative changes to allow broader biopharmaceutical company communications will help payers access information needed to inform premiums and prepare to negotiate contracts with biopharmaceutical companies and will allow providers to make more informed prescribing decisions for patients by giving them access to the most up-to-date scientific data available.

Two recent PhRMA-funded surveys of 38 payer executives from managed care organizations, pharmacy benefit managers and integrated delivery networks and 178 physicians from a range of specialties find that both payers and providers want and actively seek a range of information from innovators about their products.

Key findings of the surveys include:

1. More than 80 percent of payers and providers surveyed were interested in receiving more information from biopharmaceutical companies regarding products undergoing clinical trials in advance of FDA review, as well as information on approved medicines, for both FDA-approved uses and unapproved uses.
2. The majority of payers and providers are already actively seeking this information, which highlights that there is a demonstrated desire for these data.
3. Payers and providers recognize and agree that guardrails are needed to ensure communications are scientifically sound and support appropriate, evidence-based decision-making.
4. Payers continue to place strong emphasis on FDA approval in their decision-making.
5. Providers report that access to additional information about unapproved uses would increase patient referrals to clinical trials that may support label expansion.

These results are consistent with the core concepts set forth in the Principles on Responsible Information Sharing of Truthful and Non-Misleading Information About Medicines with Health Care Professionals and Payers jointly released by Biotechnology Innovation Organization ("BIO") and PhRMA last year (PhRMA-BIO Principles).[1]

To allow more evidence to be communicated about the value of new drugs and biologics, while at the same time providing appropriate safeguards, policymakers should look to existing, consensus-based standards and frameworks. The PhRMA-BIO Principles are one such standard. Other examples of standards defining scientifically-sound methodologies include the Good Research for Comparative Effectiveness (GRACE) principles.[2]

Survey Results: Payers and Providers Want Increased Access to Information from Biopharmaceutical Companies

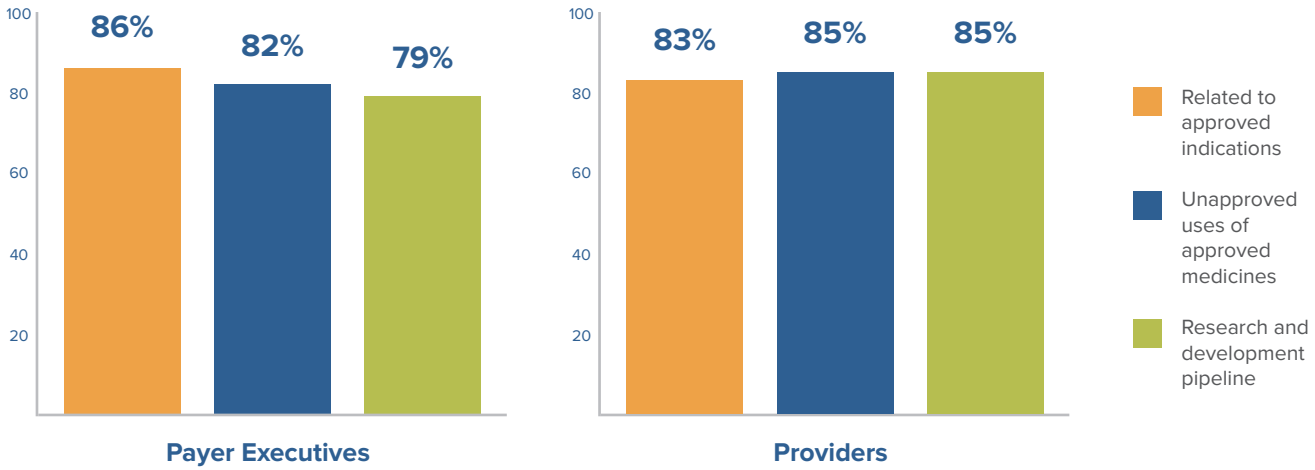
In an effort to better understand payer and provider views on the role of increased information-sharing by innovators in advancing value-driven care, PhRMA sponsored two online surveys in November 2016 with 38 payer executives representing national and regional managed care organizations, pharmacy benefit managers and integrated delivery networks and 178 physicians from a range of specialties.[3]

The surveys described payer and provider interest, perceptions and use of information across the drug lifecycle. Questions were asked about three types of information: (1) related to products in the research and development pipeline undergoing clinical trials in advance of FDA review, (2) related to FDA approved indications and (3) related to unapproved uses of approved medicines.[4]

Most Payers and Providers Are Interested in Receiving More Information from Biopharmaceutical Companies

More than 80 percent of payer and provider respondents report that it would be helpful to receive more information from biopharmaceutical companies related to approved indications and regarding unapproved uses of approved medicines. A similar proportion would also like to receive more information on products in the research and development pipeline.

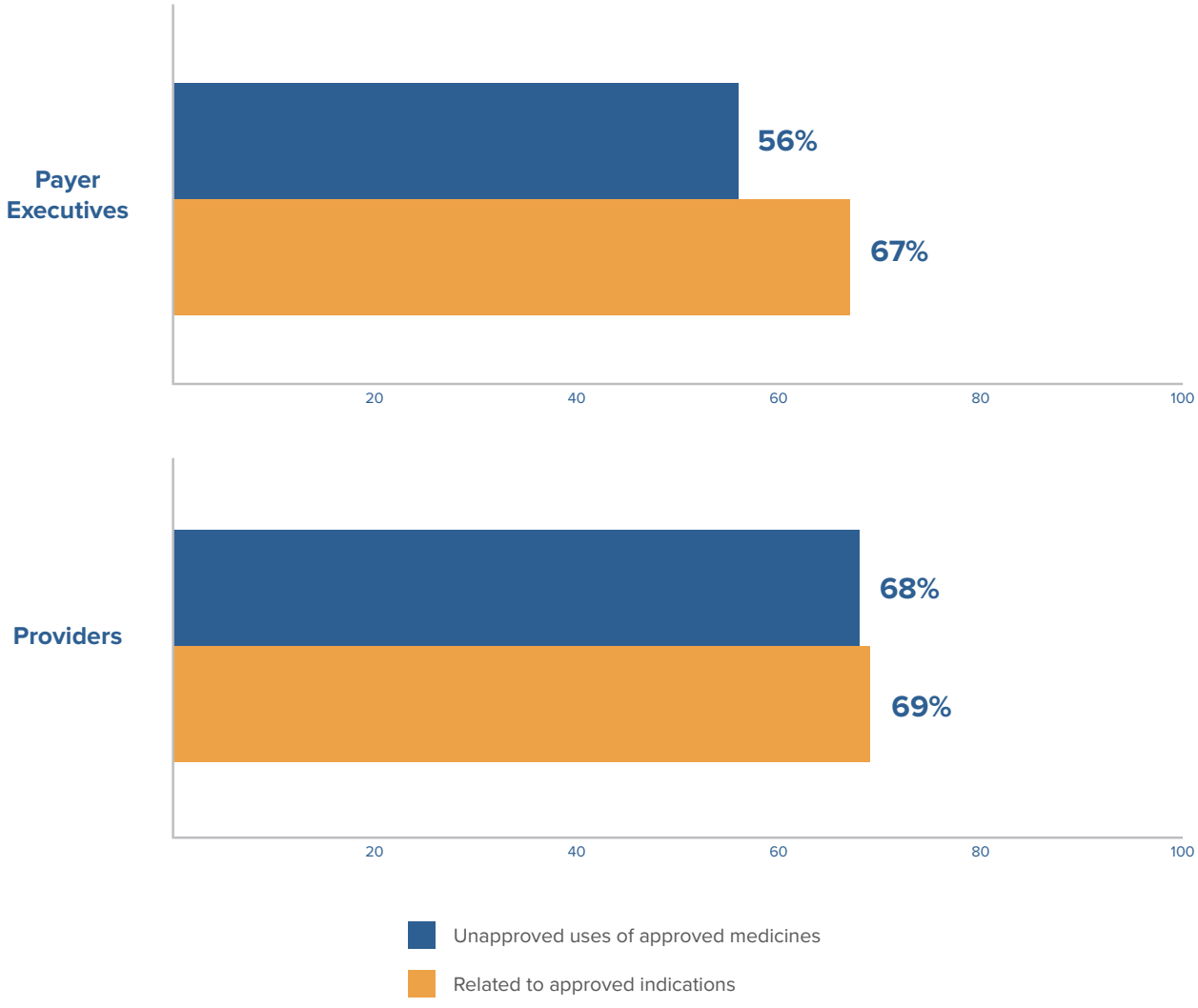
Percent of Payers and Providers Interested in Receiving More Information from Biopharmaceutical Companies



Payers and Providers Want Information on Approved and Unapproved Uses of Medicines

Payers and providers already routinely seek information from biopharmaceutical companies related to approved indications for medicines, as well as for medically accepted, unapproved uses. In fact, nearly 60 percent of payers and more than two-thirds of providers seek additional information outside of the FDA label at least four times per year. These findings demonstrate a significant demand from payers and providers for information about uses of products outside the FDA-approved labeling.

Percent of Payers and Providers Seeking Additional Information at Least Four Times per Year



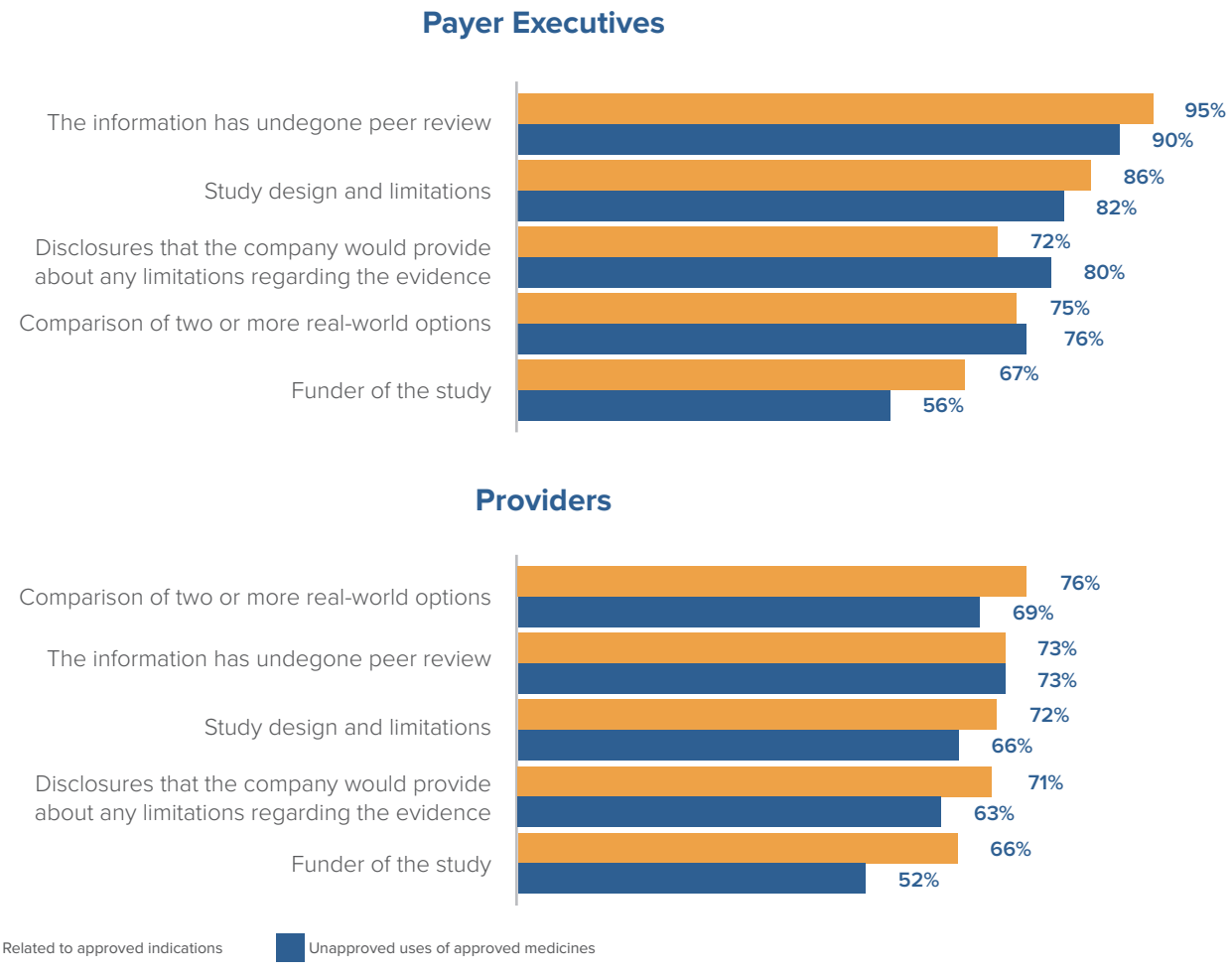
Payers and Providers Value Information that is Accurate and Scientifically Sound

According to survey respondents, payers see the most value in clinical data that has been peer-reviewed and for which information regarding study design and limitations is made available. Providers see the most value in clinical data that provides a comparison with real-world options and that has been peer-reviewed. Payers and providers are also interested in disclosures about limitations of the evidence.

These priorities align with the three core concepts outlined in the PhRMA-BIO Principles released in 2016:

1. Communications should be based on analyses using scientifically and statistically sound methodologies;
2. Communications should clearly disclose appropriate contextual information to ensure that the recipient has all the material necessary to make an informed assessment about the quality of the information presented; and
3. Communications should accurately represent the data on which they are based and should be tailored to the sophistication level of the intended audience.

Value in Evaluating the Validity and Utility of Additional Data from Biopharmaceutical Manufacturers (Percent of Respondents Indicating Element is Valuable)

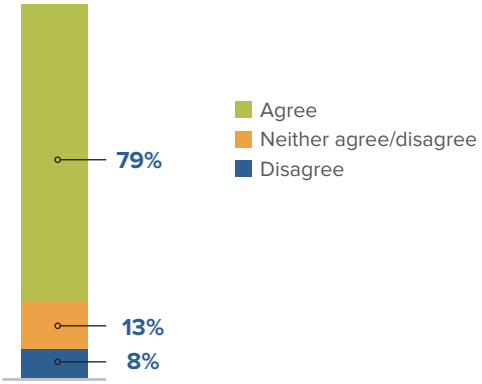


FDA Approval Will Remain the Gold Standard for Payers

Allowing biopharmaceutical companies to share more information with payers would reflect the realities of real-world payer decision-making as they seek to anticipate and account for new product approvals, and align policy with standards of care across a range of medically appropriate product uses. At the same time, the survey found that FDA approval will remain the gold standard for payer coverage and payment decisions. 79 percent of the payers surveyed said that, if biopharmaceutical companies were able to proactively communicate more information regarding unapproved uses of a product, they would still want to see the biopharmaceutical company obtain FDA approval for those uses. Providing greater clarity on innovators' ability to communicate a wider range of information to payers could incentivize more research and lead to more opportunities for additional FDA-approved indications.

Payer Executives

If companies were able to proactively share more information regarding unapproved uses of a product, I would...



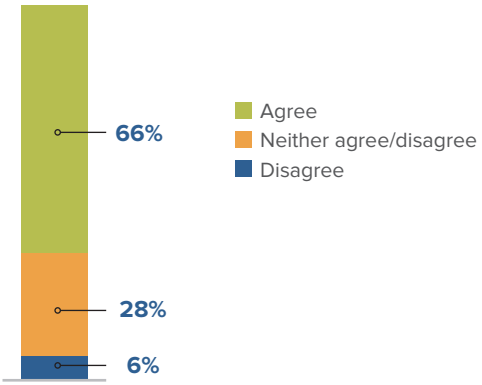
Still want to see the manufacturer take steps to have the use approved as an indication in the product labeling

Providers Say Additional Communications Will Support Clinical Trial Participation

Allowing biopharmaceutical companies to share more information with providers about unapproved uses will support clinical trial participation to study those uses. Among the 178 specialist physicians surveyed, two-thirds agreed that if they had more information about unapproved uses of medicines, they would more often refer patients to clinical trials that seek to develop evidence about the benefits of these uses. Another 28 percent indicated that additional information would not impact their referrals to clinical trials.

Providers

If I had more information about unapproved uses, I would...



More often refer patients to clinical trials that seek to develop evidence about the benefits of these uses

Discussion: Implications for the Evolution of FDA Regulation of Biopharmaceutical Company Communications

In September 2016, FDA announced it would review its regulations regarding biopharmaceutical company communications related to medically accepted, unapproved uses of approved medicines. As part of this review, FDA engaged with health care professionals, payers, the biopharmaceutical industry and other key stakeholders during a two-day public hearing in November 2016 to better understand manufacturer communications.

Discussions included the types of information that are most important to judge the validity and utility of communications about unapproved uses.

In January 2017, FDA issued a number of additional documents related to biopharmaceutical company communications about the uses of approved medicines and medicines in the research and development pipeline.

Recent FDA Documents Related to Biopharmaceutical Company Communications

<table>
<tr><th rowspan="2">Audience</th><th rowspan="2">Investigational Products</th><th colspan="3">Approved Products</th></tr>
<tr><th>Health Care Economic Information</th><th>Consistent with Approved Indication</th><th>Unapproved Indications</th></tr>
<tr><td>Payers and Population Health Decision Makers</td><td colspan="2">Draft Guidance: Drug and Device Manufacturer Communications with Payers, Formulary Committees or Similar Entities (Jan 2017)</td><td rowspan="2">Draft Guidance: Medical Product Communications that are Consistent with the FDA-Required Labeling (Jan 2017)</td><td rowspan="2">Final Rule: Amendments to Regulations Regarding “Intended Uses” (Jan 2017, Effective Date Delayed to March 2018)

Public Meeting: Manufacturer Communications Regarding Unapproved Uses of Approved or Cleared Medical Products (Nov 2016)

Memorandum: Public Health Interests and First Amendment Considerations Related to Manufacturer Communications Regarding Unapproved Uses of Approved or Cleared Medical Products (Jan 2017)</td></tr>
<tr><td>Health Care Professionals</td><td colspan="2">No Recent Changes</td></tr>
</table>

These documents provided helpful clarification to biopharmaceutical companies regarding FDA’s current viewpoint on biopharmaceutical company communications. In these documents, FDA proposes to allow certain communications based on sound science, subject to disclosure of contextual information and limitations to help ensure that the communications are truthful and non-misleading for their intended audiences.

While these draft guidances are a positive step forward, the results from the surveys discussed above reinforce the need for further changes to ensure payers and providers can receive information from biopharmaceutical companies about unapproved uses of approved medicines.

Conclusion

As these survey results make clear, payers and providers would like more information about products in the pipeline and approved and unapproved uses of existing medicines, which would be supported by updated FDA rules governing biopharmaceutical company communications. With additional information, payers can make better coverage decisions and work more closely with biopharmaceutical companies to implement new ways to pay for medicines, in an effort to ensure patient access to the newest medicines and potentially produce cost savings for the overall health care system. For providers, additional information can help them make more informed prescribing decisions for patients, taking into account the most up-to-date scientific data available. Allowing greater information-sharing will also alleviate the significant delays caused by the current system of unsolicited requests, in which information is only shared when a specific request is made by a physician.

With the right safeguards in place, removing restrictions on biopharmaceutical company communications with payers and providers can support efforts to link payment to value and to deliver more patient-centered care, while continuing to protect public health.

Sources

[1] PhRMA/BIO Principles on responsible sharing of truthful and non-misleading information about medicines with health care professionals and payers. 2016. Available at: http://phrma-docs.phrma.org/sites/default/files/pdf/information-sharing-with-hcps-principles-report.pdf

[2] Dreyer NA, Bryant A, Velentgas P. The GRACE Checklist: A Validated Assessment Tool for High Quality Observational Studies of Comparative Effectiveness. Journal of Managed Care & Specialty Pharmacy. 2016 Oct;22(10):1107-13.

[3] Detailed information on respondents shown in **Appendix Table 1**

[4] Definitions used to characterize “related to an approved indication” and “unapproved uses of approved medicines” shown in **Appendix Table 2**

Appendix

Appendix Table 1. Study Sample and Exclusion Criteria

Payer Executives*	
National Managed Care Organizations (MCOs)	10
Regional MCOs	10
Pharmacy Benefits Managers (PBMs)	8
Integrated Delivery Networks (IDNs)+	10
Total	38

*Executives were asked to choose their level of involvement in the decision-making process for their organization on a scale of 1 (Not at all involved) to 7 (I am a key decision maker). Potential participants responding below 4 on this scale were screened from the survey.

+ IDNs were required to have an active Accountable Care Organization (ACO) program, population health program, or risk program to participate in the survey.

Specialist Physicians*	
Cardiologists	20
Endocrinologists	19
Hematologists/Oncologists	40
Neurologists	37
Psychiatrists	21
Rheumatologists	41
Total	178

*Physicians were required to be in practice for between 2 and 30 years, be board certified or board eligible in their specialty, spend at least 50 percent of their professional time in clinical practice and see at least 200 patients per month.

Appendix Table 2. Definitions Used to Characterize "Related to an Approved Indication" and "Unapproved Uses of Approved Medicines"

Related to an Approved Indication	Unapproved Uses of Approved Medicines
Data on subgroups of patients within the approved population (e.g., race or gender specific information regarding a medicine that is approved in all adults)	Utilization for different diagnoses not listed in the product label (e.g., to treat schizophrenia if label is bipolar disorder)
Data on outcomes not in the product labeling (e.g., long term outcomes related to the clinical trial endpoints on the product label)	Utilization in patient populations not included in product label (e.g., to treat patients under 18, if label is in patients 18+)
Pharmacoeconomic data	Utilization in different lines of therapy than indicated
Post market surveillance data	Utilization in an unapproved regimen/combination
Comparative effectiveness data	

PhRMA
RESEARCH • PROGRESS • HOPE

HealthStrategiesGroup®