NHF News Briefs

April 20 – April 26, 2018

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This report includes selected news items from the past week on issues of concern to the bleeding disorders community. It is designed to help keep NHF national and local leadership and staff informed of the latest information from the news media. It will be distributed by email on Thursday of each week, covering important news items from the previous seven days. Subjects covered will include hemophilia, other bleeding disorders, gene therapy, hepatitis, HIV/AIDS, and others.

April 20, 2018
Infectiousdiseaseadvisor.com

Glecaprevir/Pibrentasvir Highly Efficacious for HCV/HIV Coinfection

By Zahra Masoud

Once-daily glecaprevir coformulated with pibrentasvir may be a highly efficacious and well-tolerated treatment for patients with hepatitis C virus (HCV)/HIV-1 coinfection with or without cirrhosis, according to a study recently published in Clinical Infectious Diseases.1

In this phase 3 study (ClinicalTrials.gov identifier: NCT02738138), the efficacy and safety of glecaprevir/pibrentasvir was evaluated in patients with chronic HCV genotype 1 through 6 and HIV-1 coinfection, including patients with compensated cirrhosis. From May to September of 2016, 153 patients coinfected with HCV/HIV from 9 countries were enrolled in the study. Participants were age ≥18 years and had body mass index ≥18 kg/m2. Compensated cirrhosis was defined by liver biopsy, transient elastography, or serum biomarkers and diagnosed in 16 patients. Patients were either HCV treatment naive or experienced and either antiretroviral therapy naive or on a stable antiretroviral therapy regimen for at least 8 weeks. No patients with HCV genotype 5 were enrolled.

Coformulated glecaprevir/pibrentasvir (300 mg/120 mg) was given orally as 3 100-mg/40-mg tablets taken once daily with food for either 8 (patients without cirrhosis) or 12 (patients with cirrhosis) weeks. HCV RNA plasma was collected and the level was quantified at screening, at day 1, and at weeks 1, 2, 4, 8, and 12, if applicable. The primary efficacy end point was the proportion of total patients with sustained virologic response (SVR) HCV RNA <15 IU/mL 12 weeks after the last day of treatment (SVR12).

Participants treated with once-daily glecaprevir/pibrentasvir achieved an overall 98% SVR12 rate with no relapses regardless of cirrhosis status. Patients without cirrhosis achieved a 99% SVR12 rate after 8 weeks with no virologic failures. One patient with cirrhosis who was HCV treatment naive experienced breakthrough. The low virologic failure rate (1/153; <1%) suggests that SVR12 achievement was not affected by high baseline viral load, presence of baseline polymorphisms, cirrhosis status, or other baseline factors.

Consistent with previous observations, this study showed a favorable safety and drug-drug interaction profile for glecaprevir/pibrentasvir, suggesting the regimen can be administered with minimal on-treatment monitoring. The safety profile of glecaprevir/pibrentasvir was similar in all patients regardless of treatment duration. The most common adverse effects were fatigue and nausea. Although no patient use of tenofovir disoproxil fumarate has been associated with onset or worsening of renal impairment, these effects may be exacerbated by co-administration with certain concomitant medications (HIV-1 protease inhibitors).2,3 In the current study, 87 (57%) patients were taking tenofovir disoproxil fumarate and none experienced worsening of renal function.

Overall, glecaprevir/pibrentasvir treatment yielded similarly high SVR rates as those reported in patients with HCV monoinfection. Study authors concluded that, "these results support the indication of glecaprevir/pibrentasvir as the first 8-week pangenotypic treatment option for HCV/HIV-1 coinfected patients without cirrhosis."

Disclosure: This clinical trial was funded by AbbVie Inc.

References

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2. Cooper RD, Wiebe N, Smith N, Keiser P, Naicker S, Tonelli M. Systematic review and meta-analysis: renal safety of tenofovir disoproxil fumarate in HIV-infected patients. Clin Infect Dis. 2010;51(5):496-505.
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Prnewswire.com

Prime Therapeutics Finds Costs Double When Switching to New Extended Half-Life Hemophilia Factor Products

Real-world data analysis shows extended half-life coagulation factor products bring high cost

An analysis of real-world, integrated pharmacy and medical claims by pharmacy benefit manager (PBM) Prime Therapeutics LLC (Prime) reveals the cost of coagulation factor products to treat hemophilia more than doubled for members who switched from standard half-life (SHL) to extended half-life (EHL) products. Prime researchers will present the study at the Academy of Managed Care Pharmacy's (AMCP) Managed Care & Specialty Pharmacy 30th Annual Meeting April 23-26 in Boston.

Conventional SHL coagulation (clotting) drugs prevent bleeding and are dosed two to three times per week. EHL therapies are designed to keep clotting factor circulating in the body longer, stretching the time between infusions and decreasing risk for bleeding. Based on the findings of this study, if the 80 percent of Prime's members with hemophilia A who are currently using SHL products switched to new EHL products, costs for health plans and its members could increase by more than $130 million per year. Therefore, PBMs and health plans need to closely assess EHL clinical value and cost effectiveness.

Hemophilia is a severe bleeding disorder resulting from the lack of an essential clotting protein (factor). While it only affects approximately 20,000 Americans, hemophilia demands a disproportionate share of health care dollars, primarily because the per person specialty drug therapy costs far exceed the pharmacy spending in many other chronic disease conditions.

Individuals with hemophilia A have a Factor VIII deficiency and make up about 80 percent of individuals with hemophilia, while those with hemophilia B are marked by a Factor IX deficiency, and comprise the remaining 20 percent. Clotting factor is prescribed to treat hemophilia in the form of coagulation factor products.

For the study, Prime analyzed integrated pharmacy and medical claims data for an average of 15 million commercially insured members per month from January 2013 to July 2017 to identify all hemophilia A and B members with claims for a factor product. Thirty-four hemophilia A and 20 hemophilia B members who switched from SHL to EHL therapy met the study requirement of continuous enrollment. And of these 54 members both pre- and post-switch costs were analyzed to calculate the impact of switching on both factor cost and amount of drug units used.

For the 34 members with hemophilia A, mean cost for SHL was $127,168 per six months compared with $300,429 (2.36 times higher) for EHL. Mean units per six months was 33 percent higher for EHL than SHL. For the 20 members with hemophilia B, mean cost for SHL was $116,909 per six months compared with $230,210 (1.97 times higher) for EHL. Mean units per six months was 14.9 percent lower for EHL than SHL for hemophilia B. None of the 54 members who switched therapies had claims that could be identified as indicating a bleeding event before or after the switch.

If the 80 percent of hemophilia A members currently using SHL switched to EHL, costs could potentially increase by more than $130 million, or more than $0.70 per member per month (PMPM).

"This analysis of real-world claims data found that switching to EHL products often did not reduce the number of factor units members used. The frequent use of more factor units after the switch to an EHL product and much higher per unit cost of EHL products led to substantially higher costs for hemophilia treatment," said Kevin Bowen, M.D., principal health outcomes researcher at Prime. "Use of EHL products has led to substantial increases in the cost of hemophilia treatment in this commercially insured population that need to be justified clinically."

To help manage hemophilia, Prime employs a utilization management strategy designed to appropriately manage EHL products. Prime's rigorous formulary development process provides clients and their members with formularies that are clinically complete and cost effective to help manage high specialty drug costs associated with chronic and rare conditions such as hemophilia.

About Prime Therapeutics

Prime Therapeutics LLC (Prime) helps people get the medicine they need to feel better and live well. Prime manages pharmacy benefits for health plans, employers, and government programs including Medicare and Medicaid. The company processes claims and offers clinical services for people with complex medical conditions. Prime serves more than 27 million people. It is collectively owned by 18 Blue Cross and Blue Shield Plans, subsidiaries or affiliates of those plans. For more information, visit www.primetherapeutics.com or follow @Prime_PBM on Twitter.

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Usatoday.com

Drug copay groups: Critical patient charities or fronts for drug makers?

By Jayne O'Donnell, David Robinson, Ken Alltucker and Liz Freeman

Copayment assistance groups, created to help patients with the increasingly higher price of drugs to treat medical conditions, are under investigation by federal authorities for possibly skewing the cost of health care to favor drug companies.

The investigations, noted by several drugmakers in their regulatory filings, are slowing contributions to at least two of these assistance groups, charities that sometimes pay top executives salaries of $300,000 or more.

Critics of these groups, such as Patients for Affordable Drugs founder David Mitchell, say they drive up the cost of health care by masking the price of drugs and forcing higher costs on the insurance companies that pass them along to consumers and employers. Copayments are the part of drug bills that insurers require consumers to pay to make them aware of the true cost of medication and encourage them to seek cheaper alternatives such as generic medications.

The money to pay for the groups' support of patients comes almost entirely from the drug companies themselves or other charities they fund.

"These groups are a marketing arm of pharma, and the fact that patients are caught in the middle of all this is disgusting," Mitchell says. "Patients shouldn't have to live month to month, at the mercy of the drug companies."

Supporters include Leonard Rodgers, a Tempe, Ariz., patient with an incurable blood cancer. The groups Good Days has had his share of two drugs that have kept him alive and cost more than $200,000 a year.

"If I end up paying for this, I'd have to liquidate all my assets within five years," said Rodgers, 80, who relies on Social Security and a modest income from non-profit charity work.

Another group, Patient Services Inc., was started by former pastor Dana Kuhn in 1989 to pay patients' insurance premiums. Kuhn, who has hemophilia and developed hepatitis C and HIV during a blood transfusion in the early 1980s, was struggling to pay for his own insurance at the time.

Now, however, Kuhn is making about $600,000 a year at a group that brought in $86 million last year to help 28,000 people pay for their medicine. The salary, he says, is justified because he was a CEO for 28 years and "we're really doing the work of the angels."

As he prepares to retire April 30, Kuhn, 65, is in Washington this week lobbying for legislation that would protect copay and premium assistance from insurers who have been prohibiting its use in plans purchased on the Affordable Care Act (ACA) exchanges. His story and that of the other groups in investigators and insurers' crosshairs illustrate a little-understood twist on how Washington really works.

Drugs could and should be cheaper for everyone, says Paul Kleutghen, a former pharmaceutical industry official who sold his last company to Novartis in 2003. Each $1 million industry donation that is used to help patient get high priced drugs has the potential to generate up to $21 million for the drug company, according to a report last year from Citi Research, so "you cannot call that charity," Kleutghen says.

"There ought to be something called a moral compass for those companies who make the decision to price at such a high level," says Kleutghen, who now takes $750 worth of drugs a day for his blood cancer, multiple myeloma, which is one of the costliest forms of cancer. "If they really want to be charitable, they ought to give the drugs away for free to the poor and lower the prices for all."

Kickback concerns

The U.S. Attorney in Massachusetts and the Health and Human Services (HHS) Inspector General have been investigating this insurance copayment assistance for more than three years. Kuhn and Clorinda Walley, the president of Good Days, say the investigations have cast a pall over donations and forced them to shut down funding for drug copays for several diseases.

The fracas over drugmaker Mylan's Epipen prices nearly two years ago shows what's at stake. It took high-deductible health plans, which are supposed to make people smarter shoppers, to prompt public outrage over the skyrocketing cost of the allergy medicine injectors children needed for home and school. Congressional hearings, increased copay assistance and lower-priced alternatives soon followed.

"I understand the problem, but the solution is not to take it out on patients by taking away the help," says Jackie Trapp, a former Wisconsin high school teacher who also has multiple myeloma. "Someone has to have the guts to stand up to the pharmaceutical companies and to punish the people doing the wrong stuff."

Trapp, 53, became eligible for Medicare in February when she was approved for Social Security disability coverage. After she pays $5,000 out of pocket, she will have to pay 5% of all her medication costs and will need copay help.

It's illegal under federal "anti-kickback" law for drug companies to pay patients' Medicare drug copays and any patient group that covers these copays can't steer consumers to their pharmaceutical donors' drugs. In December, the Justice Department reached a settlement agreement with drugmaker United Therapeutics (UT), which sells several drugs that treat pulmonary arterial hypertension.

The government accused UT of paying kickbacks to Medicare patients through a charity, Caring Voice Coalition (CVC), "that held itself out as an independent charitable foundation." The drugmaker paid $210 million to resolve the allegations and Justice rescinded an advisory opinion it issued CVC because the group "allowed its drug company donors to funnel money through CVC in potentially illegal ways that served the drug companies' financial interests."

This practice, the HHS inspector general said in a statement, could harm patients, the government and taxpayers because people might take pricey drugs instead of cheaper ones.

"The government took these actions to protect the Medicare program and the taxpayers who fund it from schemes like these," said the inspector general's office.

The groups say they provide assistance to patients who contact them based on need and their prescriptions, not favoritism toward their donors.

Preaching about copays

When Kuhn got a "bad infusion" while hospitalized in the early 1980s, HIV was little understood. By the time doctors knew what was wrong with him, Kuhn had infected his wife. Fourteen months later in March 1987, she was dead.

Suddenly a single father with two preschool-age children, the Presbyterian minister needed a job that had insurance. He found one counseling people with chronic diseases after he showed up at the emergency room following a construction site injury.

As insurance premiums rose, his new type of parishioners were taking drastic steps to get coverage, including one couple who got divorced so the mother and child could go on Medicaid. Kuhn came up with the concept of premium assistance and first got specialty drugstores to fund it until he branched out into copay help funded by drug makers when pricey multiple sclerosis drugs his the market in the early 1990s.

Without this, "people who make too much money to qualify for a free drug program have to deplete their savings or beg or borrow to afford their co-payments," says Kuhn. "Many of the stories are that people stop taking their drugs, their conditions become exacerbated so they go to emergency rooms where they can’t be denied their drug and can get stabilized for little or no money."

Then, Kuhn says, the cycle starts all over again.

The investigations have only made it worse, Kuhn and Walley say. PSI's funding was down 17% last year, and Walley says her funding is flat, but she had to slash the number of diseases supported. Two of the other groups under investigation say it hasn't had the same effect on them, however. HealthWell says it doubled the number of people it served last year to 90,000 and had a record year in donations. In a statement, the group attributed that to the "transparency and compliance" it has adhered to since it launched in 2003.

PANF's contributions have remained constant at about $500 million for the past two years, says CEO Klein, but it now has to shut down disease funds when they run out in the middle of a quarter or year, rather than to reliably get a new infusion when it's requested.

Kuhn says he also can't rely on getting additional money beyond what's approved in grants.

PANF received the most pharma funding of any patient assistance group in 2015, according to a new database by the non-profit Kaiser Health News (KHN) that includes 14 of 20 drug companies in the S&P 500. The analysis doesn't include all donations, every drug company or every patient group because KHN limited the scope of its analysis and companies aren’t required to disclose their non-foundation giving. Drug maker Celgene, which raised prices for its multiple myeloma drugs three times last year, refused to provide any information to KHN.

Kuhn blames the government, not the drug companies who back his group.

Meanwhile Kuhn is no longer among "the poor, the widowed, the orphaned and the sick" he purports to help, and neither are his colleagues.

PSI vice president James Wood made $320,000 and six others made more than $100,000 each, according to 2016 federal tax filings. The median income in the zip code where PSI is based in Midlothian, Va., is $83,000.

Walley isn't either. She took over as president of Plano, Texas-based Good Days — then known as the Chronic Disease Fund — in 2014 after founder Michael Banigan was forced out after reports that CDF favored Questor Pharmaceuticals and conducted millions in business transactions with for-profit companies Banigan owned.

Walley, who never graduated from college and has suffered from ulcerative colitis for years, was hired by Banigan from a specialty pharmacy where she was a technician and later director of operations. She earned $273,000 in 2016, according to tax filings.

To Mitchell, who founded Patients for Affordable Drugs last year, that's just one of the examples of what's wrong with this system of paying for prescription drugs. Mitchell, who also has multiple myeloma, can afford his Medicare cost sharing and helped fund his group along with the Laura and John Arnold Foundation, which donates to causes including drug pricing transparency.

"These people may have started out with a mission of service, but now they are making a great deal of money doing the bidding of drug corporations," he says.

Real people, problems

Rodgers, of Tempe, Ariz., says Good Days pays the travel costs for his 22-mile trip from his home to the Mayo Clinic in northeast Phoenix: “They pay just like a corporation would pay — a per-mile charge."

Good Days has encouraged him to tell others of his experience with the charitable organization, Rodgers says. It has paid for him and his wife to attend Good Days’ annual donor conference the past three years that has included appearances by celebrities including Jay Leno.

Rodgers concedes the life-saving medications are often too costly for many to afford without financial assistance. But he said drug companies must be reimbursed for the cost and risk of getting drugs approved.

“I am a little bit sympathetic with the drug companies,” Rodgers says. “People don’t appreciate the cost of research.”

Kleutghen does. His first job was at pharma giant Pfizer, which gave more money to patient advocacy groups than any other drugmaker included in Kaiser Health News database. He also worked for years at a generic drug company. Pharma could easily lower prices considerably without impacting their profitability or investment in research and development, he says.

Some health plans and other critics argue curbing nonprofits like PSI would force drug makers to reduce prices, or drive more patients to less-expensive generics and alternatives.

“It’s certainly worth greater transparency and disclosure about those programs to ensure that consumers are getting the value and aren’t nearly being directed to higher-cost drugs,” says Eric Linzer, CEO of New York Health Plan Association.

Good Days covers the $695 a month Florida Blue insurance premium and $10,000 annual deductible for Sierra Price, 15, who has the rare genetic disease, cystinosis. Good Days has even pledged to cover what insurance doesn't cover for Price's kidney transplant this summer.

“When I tell you without Good Days, she dies, it’s the truth because we cannot financially keep her alive,” says Price's aunt, Hanna Boisselle, of Naples, Fla., who handles Price's insurance and medications

The family learned about Good Days in 2013 from a sales representative for Raptor, which had just come out with Procysbi, which contains the only treatment that slows progression of cystinosis without the side effects of her previous drug, Good Days advised putting Sierra on Florida Blue insurance, and said it could pay the premium.

Every year, Florida Blue tries to say Sierra doesn’t need the more expensive Procysbi, but Boiselle says Good Days helps with the fight.

Groups like hers "are the consumer's only voice," says Good Days' Walley, but they "don't have power" to influence high drug prices, she says.

"We don't think we're the answer, but patients don't really have any other alternative," agrees PANF's Klein.

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Prnewswire.com

Patient Advocacy Groups Focus on Key Issues Affecting Patients

Five patient advocacy organizations are awarded LEAD Grants to support their advocacy initiatives in 2018.

Patient advocacy groups in Michigan, Washington, Illinois, Missouri and South Carolina are preparing to meet the many challenges standing between patients with rare bleeding disorders and access to care. Their efforts are being supported in part by Local Empowerment for Advocacy Development (LEAD) grants from global biotherapeutics leader CSL Behring.

Susan Fenters Lerch, executive director of the Hemophilia Foundation of Michigan (HFM), said her organization is using its LEAD grant to help counter what she describes as "burgeoning threats to health care." HFM's strategy is to increase youth involvement, education and leadership in state-level advocacy to protect and enhance access to care.

"Advocacy is a necessary part of life for all members of the bleeding disorders community," Lerch said. "In the words of one of our teen advocates, 'a bleeding disorder is part of my identity, and advocacy comes along with that.'"

In Washington State, people with hemophilia, von Willebrand disease and other rare bleeding disorders are dealing with increasingly complex public policies involving Medicaid, co-pays, out-of-pocket expenses and changes to the Affordable Care Act. The Bleeding Disorders Foundation of Washington (BDFW) is responding to these challenges by creating Advocacy 102 Summit Training for patients with its LEAD Grant.

BDFW is training up to 40 advocates to educate elected officials regarding health care issues. The foundation also hopes to place an advocate or advocates in a position to work on the pending federal effort to evolve the framework of the Washington State Bleeding Disorder Collaborative into a federal demonstration project.

In Illinois, the Bleeding Disorders Alliance of Illinois (BDAI) supports nearly 3,000 patients affected by inherited bleeding disorders such as hemophilia and von Willebrand disease. With help from the LEAD grant program, BDAI is putting together an advocacy campaign designed to meet the current and future advocacy needs of the bleeding disorders community in the state.

The Gateway Hemophilia Foundation (GHF) in Missouri had previously developed a state-wide Advocacy Ambassador program that prepares patient-volunteers to serve as liaisons between GHF and elected officials, and to share their personal stories with decision makers. Now, with the help of a LEAD grant, GHF is building on its Advocacy Ambassador program by providing advanced advocacy training to strengthen relationships and increase engagement with key local legislators.

Hemophilia of South Carolina (HSC) is tackling several major challenges. These involve establishing a standard of care for hemophilia and bleeding disorder patients in South Carolina, including access to Hemophilia Treatment Centers and Factor therapies as well as expanding its Advocacy Ambassador program. HSC's Advocacy Ambassadors are volunteers who help to establish and build a strong

grassroots network of bleeding disorder advocates throughout the state, creating greater awareness of the needs of patients.

HSC is also working to address continuity of care, primarily through better access to HTCs in South Carolina and to better secure access to healthcare and treatment for hemophilia patients through legislation or regulatory reforms.

"CSL Behring is committed to supporting the important work of these organizations, which play a vital role in expanding patient access to information and healthcare," said Dennis Jackman, CSL Behring's Senior Vice President for Global Healthcare Policy and External Affairs.

"This is all the more important during a time of uncertainty around access to care for patients generally, and for those with rare and chronic medical disorders in particular," Jackman added. "We are driven by our promise to strive to ensure all patients have access to the medicines and services they need."

CSL Behring has awarded over $1 million in LEAD Grants since the program was established 10 years ago. The deadline for submitting proposals for the next LEAD Grant cycle is April 30, 2018. For more information visit LEAD Grant.

About CSL Behring
CSL Behring is a global biotherapeutics leader driven by its promise to save lives. Focused on serving patients' needs by using the latest technologies, we develop and deliver innovative therapies that are used to treat coagulation disorders, primary immune deficiencies, hereditary angioedema, inherited respiratory disease and neurological disorders. The company's products are also used in cardiac surgery, organ transplantation, burn treatment and to prevent hemolytic disease of the newborn.

CSL Behring operates one of the world's largest plasma collection networks, CSL Plasma. The parent company, CSL Limited, headquartered in Melbourne, Australia, employs nearly 20,000 people, and delivers its life-saving therapies to people in more than 60 countries. For more information visit www.cslbehring.com and follow us on www.Twitter.com/CSLBehring.