

Hemophilia **OUTLOOK** Newsletter

The Hemophilia Association of New York, Inc.

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Celebrating 66 Years of Service to the Bleeding Disorders Community!

On Sunday, July 29 the Association celebrated our 66th Anniversary at a Kansas City Royals vs. Yankees game at Yankee Stadium. The men's group participated in a physical therapy program led by Dr. Michael Zolotnisky prior to the game. All attendees enjoyed great weather and delicious ball park food.

*A special thank you to all of our sponsors:
Novo, Shire, Kedrion & CSL*



HANY SCHOLARSHIP

24 applicants were awarded scholarships this year. HANY offers scholarships of up to \$7,000 to full-time students attending a college, university, graduate or vocational school. In order to qualify applicants must have, or be the child of a person who has, a genetic bleeding disorder and a registered client with HANY. Applicants must also reside within the 14 southeastern counties of New York State.

Applications for the year 2019 will be available soon. For more information contact 212-682-5510, or email tconstantine@hemophiliany.com

PATIENT CO-PAY ASSISTANCE PROGRAMS

Patient assistance programs are offered by medicine manufacturers. These programs offer assistance with co-pays and deductibles. If you would like information about these programs, please contact your hemophilia treatment center or HANY at 212-682-5510.

**IT IS MANDATORY TO REGISTER
EVERY YEAR FOR YOUR SPECIFIC
ASSISTANCE PROGRAM**

The Royal Disease: A Family History update on Queen Victoria

By: Louis P Le Guyader, Ph.D.

It is unlikely that UK's Queen Victoria was a first-time carrier of hemophilia in her family; "most likely," she inherited it. This has implications for reporting the mutation rate causing hemophilia (30% seems too high). It suggests a call out to hemophiliacs – please give deeper, broader, and more complete family histories!

The "Received Truth"

Medicine describes Victoria as a female carrier who introduced hemophilia into her family as the result of a gene mutation. Victoria herself asserted that she was unaware of hemophilia genetic trait in her family. Physicians in the 1800s, and later, neutralized her "blame" or responsibility for hemophilia by using the "mutation" tag and pointed out that she gave birth to many children before she learned about it.

"Completing" the Truth"

The Queen was the daughter of a British Prince. The unlikely monarch was a female who outlived many male descendants of the King to become Queen. She is also correctly described as her father's only child.

The more complex truth is that she was also a German princess, and one of three of her mother's children. Her mother was a widowed German Princess. She had two offspring with a German Prince, a son and a daughter. Few medical details are publicly available about the daughter's line. The son, however, is reported to have died from bleeding from his internal organs in his early 50's. He had two bleeding attacks weeks apart. The local hospital successfully stopped the first bleed, but could not stop the second; he died from "apoplectic shock," consistent with hemophilia.

Royal Secrets

The death of Victoria's half-brother occurred in the 1850's, near the end of Victoria's child bearing years. The true extent of "Victorian hemophilia" would not become evident for nearly 50 years when a spate of male descendants, European royals, succumbed quite publicly.

The family situation of Victoria's German siblings masked the condition. On the son's side, no others had hemophilia and his line died out. The half-sister's line continues. At the time, the label "hemophilia" was still new, and was controversial. The collaboration between the three leading medical communities of the day in America, the UK and Germany was spotty, and this would last past WWII.

Information about Victoria's hemophilia was managed for political reasons. The hope was that her female descendants would prove attractive spouses for all European royal families, and hemophilia interfered with that agenda. The British royal family also tried to distance itself from Germany, despite several family ties; this cut off information flows.

Implications

Giving family histories further back than two or three generations helps unveil hemophilia and avoids the "first mutation" tag. Broadening histories across collinear lines, aunts, uncles, and cousins is key. The high figure of 30% of hemophilia cases described as first-time mutations likely reflects incomplete family histories.

Women should be vigilant about this! Victoria's mother was possibly a female carrier with inherited hemophilia. Her female descendants continue to be born to this day in unbroken royal female lines directly back to the Queen. Hemophilia can persist for generations without symptoms and is sometimes forgotten, through sequential female carriers, needs careful

understanding. My research and outreach includes recent contacts with members of Victoria's family including living members of her "nearly unknown" German line. Stay tuned!

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NY Makes It Easier To Swap Prescription Opioids for Medical Marijuana

By: James T. Mulder of Syracuse University

Syracuse, NY – New York is making it easier for patients who use prescription opioids to replace those painkillers with medical marijuana.

The state Health department said it has filed emergency regulations that make any condition for medical marijuana, severe pain is not. The change means people prescribed opioids for severe pain can now replace those drugs with medical marijuana.

The regulation also allows people being treated for opioid addictions to use medical marijuana as a replacement.

"Medical marijuana has been shown to be an effective treatment for pain that may also reduce the chance of opioid dependence," Dr. Howard Zucker, the state's health commissioner, said in a prepared statement.

Opioid replacement joins 12 conditions that qualify people in New York for medical marijuana. Those conditions include: cancer, HIV or AIDS, amyotrophic lateral sclerosis, (ALS), Parkinson's disease, multiple sclerosis, spinal cord injury with spasticity, epilepsy, inflammatory bowel disease, neuropathy, Huntington's disease, post-traumatic stress disorder and chronic pain.

There are more than 62,000 patients in New York certified to use medical marijuana and 1,735 providers registered to prescribe it.

Health-care, Pharmaceutical Providers Testing RFID to Track Hemophilia Medication

By: Clare Swedberg

Specialty pharmacy US Bioservices is piloting an in-home inventory management program using RFID technology, with MedImpact Healthcare Systems, for monitoring the at-home use of medications by patients with hemophilia, a genetic disorder impairing the body's ability to form blood clots. The system, consisting of passive UHF RFID (Ultra High Frequency Radio Frequency Identification System) enabled coolers that track, in real time, which medications are being stored and which are being used, is intended to prevent the overstocking or expiration of medications, while helping health care providers understand and manage each patient's condition. The technology is provided by the US Bioservices' parent company, AmerisourceBergen.

US Bioservices provides patient support, including specialty medication and clinical care. Among its customers are patients with hemophilia who require drugs to manage their condition. Hemophiliacs typically live normal, healthy lives, but they require medication either as prophylactic or when an injury occurs that could lead to bleeding. Typically, they keep these medicines on hand, since the drugs may need to be used on an emergency basis.

Like other medications, hemophilia drugs can be high-priced and have a specific shelf-life. They also require refrigeration to be effective, says Kevin James, US Bioservices' payer strategy VP, so tracking them is critical- though this can be difficult to accomplish when the drugs are dispersed to the homes of patients who may or may not need to use them regularly. Patients typically store the medicines in their home refrigerators, making it easy to lose track of them or enable them to expire.

MedImpact Healthcare Systems is a pharmacy benefit manager (PBM) that provides home delivery of drugs for patients' health insurance companies. The system being piloted is part of the firm's MedImpact Direct Specialty Program pharmacy delivery service.

When a drug is placed inside the cooler, the reader captures the ID number encoded to its tag, then forwards that data to software hosted on a cloud-based server, via a cellular connection. The software links the medication with the cooler, along with the individual using that device. In that way, the patient, the health-care provider and the pharmacist can each view what medication is being stored there, as well as whether it is nearing expiration. Each time an individual removes a drug from the cooler, he or she must first provide information to the system. The system is intended to enable US Bioservices and MedImpact to determine when a patient may require a medication refill. Analytics from the data will also make it possible to identify whether a patient has more medicine on hand than he or she needs- for instance, if that person uses medication at a lower rate than anticipated.

The pilot, which began in July of this year, will continue for at least six months before being assessed, James says. The participants will assess the return on investment (ROI) the system provides, based on the reduced risk of medication expirations or over-stocking.

Thoughts...

- Will the future model replace Doctors and nurses who maybe outside this "loop" of information?
- Will cloud-based information be 100% safe from hacking?
- Will this lead to more oligopolies?

Source:

<http://www.rfidjournal.com/articles/view?17709>

One-Third of Chronic HCV Patients Get Denied Direct-Acting Antiviral Therapy

Four years after the first drugs capable of curing chronic Hepatitis C (HCV) infection hit the market, one-third of patients prescribed direct-acting antiviral therapy are denied coverage by their insurance companies.

That's the finding of a new study based on data from a specialty pharmaceutical firm, including records of more than 9,000 patients.

Overall, 33.5% of patients prescribed a direct-acting antiviral (DAA) regimen for chronic HCV were denied. Among those patients with private insurance, the rate increased to 52.4%. Patients on Medicaid were denied 34.5% of the time, while patients on Medicare had a lower denial rate of 14.7%. The study data cover the period from January 2016 through April 2017. Over that time frame, the data show denial rates were increasing.

"I think these denials are due in part to the high costs of the HCV antivirals and insurers concerns regarding the costs for these drugs inflating their budgets," senior author Vincent Lo Re III, MD, MSCE, an associate professor of medicine and epidemiology at the University of Pennsylvania's Perelman School of Medicine, told MD Magazine.

But Lo Re said the problem also has to do with the fact that the drugs are being prescribed to more patients with chronic HCV who have less advanced liver fibrosis.

"These patients are coming up against insurers' restrictions to access unless patients have more advanced liver fibrosis," he said. Those restrictions run contrary to the recommendations of both the Infectious Diseases Society of America and the American Association for the Study of Liver

Diseases. Both organizations recommend that all patients with chronic HCV infection be treated with direct-acting antivirals.

Though the drugs are expensive, Lo Re said the data — both clinical and financial — support the use of these medications. He noted that left untreated, chronic HCV patients are at risk for a host of other complications.

“Cost-effectiveness analyses have demonstrated that these new hepatitis C treatments are cost-effective even at early stages of liver fibrosis,” he said. “When you factor in the costs of liver transplantation, hospitalizations to manage complications of liver failure, and treatment/management of HCV-associated liver cancer, the downstream costs of these issues far outweigh the up-front cost of hepatitis C treatment.”

He also noted that lack of treatment contributes to HCV transmission.

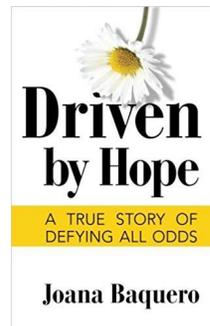
In fact, the problem of HCV under-treatment is even worse than the insurance denial data suggest, since the insurance study only reflects patients who have sought treatment. Overall, including patients who haven’t sought treatment or don’t know they have the infection; fewer than 1 in 10 chronic HCV patients in the US receives treatment. Fixing the problem, Lo Re said, will require a joint effort.

“I think it will take a coordinated effort between stakeholders in industry, government, patient advocacy groups, patients, clinicians, health services researchers, and policy makers to discuss the issue, recognize the cost-effectiveness (shown in numerous studies), and come to common ground to increase access to these drugs,” he said.

Source: <https://www.mdmag.com/medical-news/onethird-of-chronic-hcv-patients-get-denied-directacting-antiviral-therapy>

ANNOUNCEMENTS

The Association has awarded a grant to Joana Baquero to cover the translation of “Driven by Hope” into Spanish in support of our Latin American Community.



Expected Publication Date: October 2018

HANY UPCOMING EVENTS

October 20 - Blood Sisterhood
Paint Party at Muse

October 27 - White Post Farms Fall
Festival

November 3 - Blood Brotherhood at
SPYSCAPE

December 7-9 - Steven L. Margolies,
MD Family Retreat at Mohonk
Mountain House
*Current Supporters: Genentech, CSL,
Pfizer*

***POSTPONED* Spring 2019**

S.A.I.L (Self Advocacy,
Independence & Leadership)
Retreat with GUTMONKEY
(Ages 13-18)
*Current Supporters: CSL, Genentech,
Shire*

HANY's RESOURCE CENTER

MISSION STATEMENT

The mission of the Hemophilia Association of New York is to provide information, education, advocacy and direct assistance to and on behalf of people with bleeding disorders, and to encourage and support scientific research to improve medical treatments and develop cures for hemophilia and related disorders.

About this Newsletter

The Hemophilia Outlook has been around since 1952. It is produced quarterly and distributed to all the members of the bleeding disorder community.

Electronic versions of our newsletters are available on our website.

HANY does not release any personal information without consent.

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Resources Information

Hemophilia Federation of America

800-230-9797

www.hemophiliafed.org

National Hemophilia Foundation

800-42-HANDI

www.hemophilia.org

Coalition for Hemophilia B

212-520-8272

www.coalitionforhemophilia.org

HEMOPHILIA TREATMENT CENTERS

New York Presbyterian

www.cornellpediatrics.com

Mt. Sinai Medical Center

www.mountsinai.org

Northwell Health (formerly LIJ)

www.northwell.edu

Albany Medical Center

www.amc.edu

Montefiore Hospital

www.montefiore.org