Ron Katz CMS Presentation:
In May 2021, I went to my doctor for an annual physical. I walked in feeling healthy. Except for a swollen lymph node on my neck everything was just fine. Until it wasn't. That lymph node, as it turns out, was a symptom, which would be confirmed over the coming weeks, of early-stage Chronic Lymphocytic Leukemia.

But "its OK," everyone said, because it's the "**good**" kind of cancer! Really – is there such a thing? Like angina is the good kind of heart disease because it doesn't kill you right away?

In the time since that diagnosis, I have learned a lot about the disease that I will host for the rest of my life. Yes, there are treatments, and there are even people who live many years without needing treatment. Testing has confirmed that I will not be one of those people. I will need treatment soon.

Before all of this, I created a retirement plan to last me into my 90s. Why not – I was as healthy as anyone I knew. But that's 30 years away. And while the treatment options now are better than they were 15 years ago, no one knows how these medications will work over a 30-year period.

So what do we know? The demographic for CLL falls heavily into Medicare. It's clear that CLL patients relying on a Part D drug will hit the new out-of-pocket maximum and will benefit from the IRA's new payment plan option. But negotiating prices down will have zero impact on what CLL patients pay for their treatments. The Medicare program will save money and beneficiaries may find that premiums are lower – those are legitimate objectives. As a patient, though, I have to hope CMS will ensure that the cost savings are achieved **without impacting CLL patient access to current medications or discovery of new treatments.**

For the thousands of patients now doing well on one existing drugs approved for CLL, any chance that plans would require them to switch to an "alternative therapy" is an absurd risk. CLL is unlike many other diseases in that it presents itself very differently in each patient. This is why a diverse array of treatment options is essential. Patients like me know that our lives literally depend on being able to receive the right medication for our unique case, and there is no single right answer that works for all of us.

There is a bright side, though. Research on combining BTK inhibitors with venetaclax, for example, opens new possibilities for shorter treatment durations. And Jaypirca, which was recently approved for treatment of mantle cell lymphoma, is being studied for CLL. This research is costly, though, and is less likely to occur when the result would be rapid selection for price negotiation.

So I, and thousands of others like me, wait, and hope for progress. The drug development process takes so long, and many simply do not have that much time. But for the 18,000 people who are newly diagnosed each year, we must continue to offer hope. Hope for better treatments, hope for a cure, and hope that they can live out their lives.

Carly Boos CMS Presentation:

Thank you for the opportunity to contribute to the information considers in negotiating a Medicare price for Imbruvica. I'm Carly Boos, and am the Executive Director for CLL Society.

We are focused on advancing effective care for chronic lymphocytic leukemia as well as related blood cancers. I'd like to acknowledge that our patient communities have experienced dramatic improvements in their prognosis over the past decade as new treatment options have emerged. It's important to stress, though, that we are far from "mission accomplished" on addressing the unmet needs of our patient communities. As we look toward reducing the cost of drugs to the Medicare program, we urge CMS to proactively consider the downstream impacts of cost containment on research and development of new therapies in these rare cancers as well as new uses of existing treatments. We also strongly urge CMS to acknowledge that its decisions and the resulting Part D plan responses will impact cancer patients. Our patients are relying on CMS to proactively protect access to all effective treatment options.

As you know, Imbruvica was the first BTK inhibitor for CLL, and patients have benefited from having an effective treatment that they can take at home. Like most CLL treatments, it was first approved for a related B-cell cancer - previously treated mantle cell lymphoma (MCL). Although the approval in previously treated CLL/SLL followed shortly thereafter, it took 2 and a half years from initial approval to get an indication for Imbruvica as first line therapy in CLL/SLL. Like most cancer treatments, research on Imbruvica was far from over once it got FDA approval. This type of post-approval research is crucial in getting us closer to effectively treating cancers particularly those that, like CLL, are now treated as chronic diseases with patients moving through lines of therapy.

One of our biggest initial concerns with the IRA drug negotiation program as a double-edged sword was that it reframes the cost/benefit equations that drive research toward or away from a product candidate or its use in a particular cancer. We also fear that it will become increasingly difficult for manufacturers to justify post-approval research and development. If the IRA had been in effect years ago, would we have the tablet formulation that enables patients taking proton pump inhibitors to also take Imbruvica? Would we be close to realizing the potential of combining a BTK inhibitor with venetoclax and anti-CD20 monoclonal antibodies? HCL is a rare B-cell malignancy with an unmet need in patients failing to benefit from purine nucleoside analogs (PNA). A recent phase 2 study of Imbruvica in refractory Hairy Cell Leukemia has shown promising results in a difficult-to-treat population – we hope this research will eventually bring a new approved option to HCL patients, but it's unclear whether pursuing that approval would result in CMS initiating price re-negotiation and an additional level of required discounts for Medicare.

As CMS comes to a decision on what constitutes a maximum fair price, we strongly urge it to fully consider the research costs leading to additional formulations that have enabled treatment use for a broader range of patients as well as those leading to labeled and medically accepted off-label uses. Since the majority of CLL patients are or soon will be Medicare beneficiaries, and patients newly started on a BTK inhibitor are increasingly prescribed one of the newer treatments, we expect that price negotiations will directly impact the entire class sooner rather than later. The precedent CMS sets with Imbruvica will likely challenge the financial feasibility of research across the class, including treatment combinations in CLL and other blood cancers, and deprive patients and their clinicians of scientific knowledge that might improve both quality of life and survival.

With respect to access to all effective treatment options, we expect CMS has heard from patients using other selected drugs or their therapeutic alternatives. Again, I would like to emphasize that the concerns are qualitatively different in the context of a chronic cancer like CLL. Patients cannot simply switch from a BTK inhibitor that they have successfully used for months or years to another one. For the individual patient, there are no therapeutic alternatives – patients remain on their prescribed treatment until they are unable to tolerate it or their cancer progresses. If plans limit options to capsules for any of the BTK inhibitors, patients relying on proton pump inhibitors will, in effect, be denied treatment. And patients cannot "step" through failure on a preferred BTK inhibitor and expect that another approved product in the class will work. It likely won't, and the time before the patient runs out of options will be dramatically shortened. Plans cannot be allowed to implement new formulary or utilization management tools restricting access to all formulations of all BTK inhibitors. If CMS takes a passive role and simply monitors formularies over time it will put patients at risk of potential harms that cannot be undone. We strongly urge CMS to take a patient-centered, proactive approach to ensuring continued access within classes of drugs containing a selected drug.

I appreciate having the opportunity to speak to the concerns of individuals with CLL and related blood cancers and would be happy to answer any questions or provide additional information to CMS.

Robyn Brumble CMS Presentation:

Thank you for the opportunity to contribute to this important discussion on Ibrutinib and therapeutic alternatives. I will be offering my perspective as both a registered nurse and professional patient advocate of nearly 25 years, and within my current role as Director of Scientific Affairs and Research for CLL Society. My participation in this listening session and the content of my statement is not influenced in any way by industry support.

At CLL Society, one of the things I am responsible for is fielding thousands of questions per year from patients through a program we offer known as Ask the Experts. This service allows patients and their care partners the opportunity to submit questions about their disease and medications.

What I have consistently observed is that patient concerns are pretty universal, in that they all want access to the best treatment possible that will result in them being able to live their lives to the fullest.

Patients also trust that their healthcare providers will be able to prescribe the best treatment for them when it is their time to start a new treatment. Most patients are completely unaware that the treatments selected by healtchare providers are often driven-at least in part-by policies, cost considerations, and utilization management strategies.

I have heard over and over from our patient community that having access to an oral blood cancer medication has been life-changing for them. When Ibrutinib was first launched years ago, chemotherapy was one of the only options. Patients had no oral therapies to choose from, even as an off-label use.

We now know that a significant number of our patients have been able to remain on Ibrutinib for years without disease progression. But over the years, there has also been a significant proportion of patients that have had to either reduce their dose significantly or completely discontinue the drug due to unmanageable side effects or disease relapse.

If I were to think of one thing CMS can do for all of those with CLL and SLL, it would be to make sure that treatment decisions are not driven by formularies or step-therapy protocols, but rather through shared decision making between the healthcare provider and patient.

Healthcare providers already have a tremendous administrative burden due to the many documentation hurdles required to ensure that their patient's treatment selections are driven by the unique needs of each individual patient's disease. They may decide NOT to jump through the additional documentation hoops that would be required for their patients to gain access to the drug that they believe will best treat the blood cancer. As CMS has repeatedly acknowledged, healthcare provider time best spent on caring for patients.

Right now, those with CLL and SLL can decide between several treatment options that will work best for their form of the disease. That would NOT be the case if Part D plans begin to restrict coverage or limit access to the other two BTK inhibitors that are currently available.

Also, my fear is that even more of our underserved patient populations might fall through the cracks if the plan's decision stands as is, resulting in lack of access to the treatments that will be best for them.

I sincerely hope that CMS will take the extra measures that are necessary to ensure that Plans do the right thing for patients, and that CMS quickly holds Plans accountable if they do not.

Thank you for the opportunity to speak.