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Enhancing Diagnosis, Treatment, and Outcomes in Paroxysmal Nocturnal Hemoglobinuria with Novel Oral Therapeutics

Announcer:
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Dr. de Castro:
Hello. I am Carlos de Castro. I'm a Professor of Medicine at Duke University Medical Center in the Division of Cellular Therapy and Malignant Hematology.

Today, we're going to be talking about PNH. PNH is just this fascinating disease, and now, with a bunch of new treatments that have arisen, it's a good time to be talking about this very rare disease. It's estimated that about 1 to 1.5 cases per million people is the incidence rate. It's a very unusual disease. It is acquired; you're not born with this. And it's characterized by one of three different clinical pictures, either there's intravascular hemolysis, bone marrow failure with cytopenias, or thrombotic events, and it can be any or all three of these. There is incredible clinical heterogeneity amongst patients with PNH. Some are very asymptomatic. Others are just incredibly symptomatic. And it's fascinated hematologists for a long, long time.

The reason PNH is so rare is that it takes two events to likely cause it. And they can occur in either order. The first event is there has to be a mutation in the what's called the *PIG-A* gene, which is the first step in making your GPI anchor on hematopoietic stem cells and all their progeny. That is not an oncogenic mutation. Those cells have no growth advantage. So the second event has to be a condition that allows this mutant cell to become the dominant cell in the bone marrow. And we feel this is likely a T cell autoimmune attack, based on evidence both from aplastic anemia, to which this disease is highly related, and also some other research evidence.

Again, the mutation is in the *PIG-A* gene. It is acquired during normal hematopoiesis, when stem cells divide. The reason the *PIG-A* gene is involved almost 99% of the time or higher, is that it is on the X chromosome, so it only takes one single hit to knock that gene either out completely or severely down. Females have lyonized one of their X chromosomes. Males only have one X chromosome. In the other cases of all the other genes, and there's at least 20 other genes involved in making the GPI anchor, they're all on somatic chromosomes, so it would take two hits to knock them out.

When you knock out that gene, you get a reduction in the GPI anchor and any proteins that are linked to the surface of the cells on that. There are a lot of different proteins, but the two that we know are the most important, at least in PNH, are CD55 and CD59 which protect the red cells from activated complement.

As I mentioned, something has to allow the cell to mutate it to become clonally expanded. It can be either extrinsic factors, such as immune factors, and/or intrinsic factors. Some patients have secondary proliferative mutations, such as JAK2, that may drive clonal

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expansion.

And then you get what's called the PNH clone in the bone marrow. And this can involve all of your cells, granulocytes, monocytes, erythrocytes, B and T cells, natural killer lymphocytes, all of them can be involved. And we measure the clone size in the bone marrow by measuring peripheral blood, either granulocyte or monocyte clone size, using flow cytometry.

This is just a schematic showing you the overview or the synthesis of the GPI anchor.

So with the absence of CD55 and 59, whenever complement is activated, and realize it's always on to a low extent through a process called tick-over, the red cells can be lysed. And they will be lysed through the complement pathways, whether that's the alternative pathway or whether that's classical pathway. Most often it's the alternative pathway, unless they have an infection, that converges on the C3 convertase. C3 is cleaved, C3b goes on to cleave C5 into its active forms. And this forms what's called the membrane attack complex, or MAC, with C5 through C9. That forms a pore in the cell, usually the red cells, which leads to the lysis of the red cells. If it's risk enough, you will get hemoglobinuria, spilling of hemoglobin into the urine, not hematuria. The hemolytic anemia is Coombs negative. It's important to stress that. You can have inflammation from C5a, and likely the thrombotic episodes are related to that. The free hemoglobin that's released will cause smooth muscle dystonias, because it will squelch nitric oxide out of this blood system; that leads to esophageal spasms. Can have severe chest pain. You feel like you can't swallow. In males, they'll get erectile dysfunction. Abdominal pain and chest pain are very common, as is fatigue.

So just in this cartoon, you see that normal red cells are protected from complement activation because of CD55 and CD59, whereas PNH red cells don't have that protection, and they will lyse whenever a complement is activated. That lysis will lead to anemia. And not only that, it will lead to hemoglobin which will squelch nitric oxide, leading to the smooth muscle dystonias we just mentioned, the hemoglobinuria, the fatigue. Long term, people can get pulmonary hypertension, renal failure, and we think that it, in part, plays a role in thrombotic events. All of these can have a significant impact on quality of life, and, more importantly, on survival.

I mentioned before that we think that there's a T cell-mediated attack as the second step that has to occur. That can occur before you even have a PNH mutation, that there is some attack on the bone marrow, mediated by the T lymphocytes, similar to what we see in aplastic anemia.

So the clinical picture of PNH is as follows. You get hemolysis due to complement activation. From that, you get anemia and fatigue. You get hemoglobinuria. Acutely, you can have kidney damage if that hemoglobinuria is severe. Chronically, you can get kidney damage from the deposition of hemosiderin in the kidneys. The nitric oxide trapping or squelching leads to all sorts of symptoms. There can be thrombosis, usually in unusual sites, such as the liver, the spleen, the mesenteric vessels, or it can be typical DVTs or even arterial clots. And then you have the bone marrow failure with decreased blood counts or cytopenias, which is not too uncommon in PNH.

Shown here are just the clinical signs and symptoms that we see with PNH. They can be very heterogeneous. Fatigue is probably the most common symptom we see, and that's a very subjective symptom, as you all are aware. Hemoglobinuria, at least in this one registry study was only in 26% of patients who presented. Now this was out of Asia, where bone marrow failure is more common. So hemoglobinuria, we estimate to be at least in 40% of patients when they present.

And you can see here, PNH was not a good disease to have prior to our current treatments, with average survivals that ranged from 13 to 17 years, depending on which study you looked at. And since the median age of diagnosis is in the 30s, it was never a great disease to have when you tell a 30-year-old you may live on average 15 more years. The problem is the only curative therapy was a bone marrow transplant, which itself carries a 20% mortality risk, and it's hard to want to transplant somebody who may be asymptomatic and doing well and may live 13 or 15 years. So we always had difficulty with recommending transplant for these patients, and we don't do that anymore, now that we have complement therapy, as I'll show you.

The diagnosis of PNH was always tricky. Because it's such a rare disease, most doctors have never heard of it, or if they had a course in med school, they may have mentioned it. They don't remember that at all because they've never seen a case. Less than 40% of patients actually receive a diagnosis within 12 months of symptoms, and up to 24% take 5 years or longer from the time symptoms are occurring. It is primarily a clinical diagnosis, which is confirmed by a blood test. You send off for what's called a PNH screen, as we'll talk about later.

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The signs and symptoms are obviously highly variable and can mirror other conditions. Fatigue is incredibly common with so many things. Fatigue in PNH is not the first thing that comes to mind. Clinical indications of hemolysis include an elevated reticulocyte count, elevated LDH levels, reduced haptoglobin levels, elevated bilirubin levels. If a urine hemosiderin test is done, it's positive in intravascular hemolysis. We can also do a urine or serum free hemoglobin, and that's positive if it's intravascular also.

Just to recall about the types of hemolytic anemia that occurs, there can be intrinsic defects to the red cells. This includes congenital diseases such as hereditary spherocytosis, where you have a membrane or cytoskeletal abnormality, enzyme defects such as G6PD deficiency or pyruvate kinase deficiency, hemoglobinopathy, such as thalassemia or sickle cell. And then the only one that is an acquired disease in this list is paroxysmal nocturnal hemoglobinuria, which we're talking about.

Extrinsic to the red cell, you can have both immune and non-immune causes. Non-immune causes include things such as DIC or TTP, artificial valves, especially when they're leaky, or immune causes, of which warm antibody immune hemolytic anemia is the most common form of hemolysis that we see, and those are patients who are Coombs positive or DAT positive.

The diagnostic test for PNH, as I mentioned, is a blood test. It should not be done on bone marrow because of nonspecific binding of antibodies. You test both white cells and red cells, in part because the red cells are lysing rapidly, you may not detect the disease. We use monoclonal antibodies against GPI-anchored proteins such as CD59 or CD55. And what we're looking for are cells that are missing GPI-anchored proteins. And you see here in Panel A, that's a negative control antibody in that left-hand panel, where we see just this blank line. And here is a normal patient who has all of their red cells expressing CD59. Panel B shows a typical patient with PNH, where they have a population of cells that is completely missing CD59. And anything above 1% is considered PNH by diagnosis, whereas they have a normal population of red cells that are expressing CD59. Panel C shows a patient who has partial deficiency, what we call type II cells, in CD59. And then Panel D is a patient with a biclonal population of cells, one that is completely lacking type III and one that is partially lacking type II.

The FLAER test is another assay that's done using the same sort of flow cytometry panel. They have taken the aerolysin protein from the Aeromonas bacteria and labeled it with a fluorescein label, and it binds directly to the GPI anchor itself. So you're not looking for a specific antigen, you're going right for that GPI anchor. And because of that, you get a higher signal of the noise ratio and a higher sensitivity and specificity. But it can only be done on the white cells. The red cells have non-specific binding to this protein, so you can't use it there.

Now, let me just deal with clone size briefly. About 1/3 to 40% of patients have classical PNH with clone sizes that are usually larger, usually greater than 50%. And that results in a lot of hemolysis and increased thrombotic episodes. And these are the patients that we need to treat with complement inhibitors. The rest of the patients have either PNH which overlaps with a bone marrow failure syndrome with cytopenias; they will not benefit from complement inhibitors, because that's a T cell-mediated process, and treatment should be directed using immunosuppressive therapy. These patients still can clot, and some of these patients may still have hemolysis, and if detected, they will benefit from complement inhibitors. They typically have smaller clone sizes, less than 30%, and there are some patients who have what's called subclinical PNH, where your clone size is less than 10%, and you really have no symptoms and no hemolysis, and they don't benefit from any treatment at all.

PNH clones do occur in bone marrow failure, such as MDS or aplastic anemia, usually with small clone sizes. They may evolve into true hemolytic NH and the clone size may increase. Very, very small polyclonal PNH populations can be detected in normal individuals. Presumably, they've not had anything that allows these cells to become the dominant cell in the bone marrow, and in many cases, these spontaneously will regress and go away.

Who should be screened for PNH? Patients who have the acronym, CATCH, which comes from my Canadian colleagues, with unexplained Cytopenias; Aplastic anemia or a history of aplastic anemia, and these patients need to be screened yearly; unexplained Thrombosis, especially in a young person, and especially if it's in an unusual site; patient with Coombs-negative hemolytic anemia should be screened; and patients with Hemoglobinuria should be screened.

We're going to move now to treatment, which is the most active area, as we now have six drugs that are FDA approved for complement inhibition in PNH, and they're listed there. There are more in clinical trials, which is hard to believe that we still need more drugs, but there is room for improvement in treating PNH.

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Supportive care is always important in these patients. If they need a blood transfusion, you give them a blood transfusion. If they need iron, you give them iron, etc.

The role for prophylactic anticoagulation in PNH is very unclear. What we learned before complement inhibition is that anticoagulants really didn't work well in preventing clots, once you'd had a clot, so putting them on prophylaxis may just increase their bleeding risk without giving them any benefit.

Allogeneic stem cell, which is the only curative therapy for this disease, now has a very limited role, in part because the survivals have improved so dramatically with complement inhibition. Really allogeneic transplant is only used for patients with severe bone marrow failure that doesn't respond to immunosuppressive therapy.

The treatment goals now are to correct anemia, reduce fatigue and other symptoms, certainly reduce the risk of thrombosis and of all the long term complications of PNH.

We can divide our complement inhibitors now into two groups. The first group is the distal or C5 inhibitors, which were started back in 2007 is when eculizumab was approved. It targets C5; it's a monoclonal antibody that's been humanized. It was given initially as a weekly loading dose for 4 weeks and then moved on to intravenously every 2 weeks. It was approved in 2007 for adults with PNH and made a dramatic, dramatic improvement in these patients, as I'll show you in the next slide. The other two drugs, ravulizumab, is a modified form of eculizumab with a longer half-life. It was approved in 2018. And then this last year, crovalimab, which binds to a separate epitope or a different epitope, was FDA approved, and it's given subcutaneously every 4 weeks.

It, in many studies that were done, significantly reduced hemolysis within 2 weeks of giving the drug. LDH levels fell dramatically, as shown in this diagram, to near, but not quite normal levels, and we'll come back to talk about that. So there was an 86% reduction in hemolysis, as measured by LDH levels, a 92% reduction in thrombotic events, which is very dramatic, not 100% but very close to it, a 73% reduction in need for transfusions, marked improvements in fatigue and quality of life measures, and the adverse events were very similar to placebos. This drug does not treat PNH-associated bone marrow failure and usually does not completely restore hemoglobin levels, as we'll show you.

One thing we learned with the dramatic drop in thrombotic events, which used to be the number one cause of death in PNH patients, was the marked impact in survival in these patients to near normal levels, as shown in this study by Richard Kelly that was published in *Blood* in 2011. And several other subsequent studies have shown this same effect on survival.

All these drugs, because they inhibit complement C5, carry a black box warning that you need to vaccinate these patients against meningococcus, and we'll come back to vaccinations later on. The black box warning says you should vaccinate at least 2 weeks prior to receiving the first dose of eculizumab. You revaccinate according to current medical guidelines for vaccine use, which at least is every 5 years. And you'd educate these patients and give them a card to carry for warnings about meningococcal infections. So if they present to their ER, they can give it the doctor who may have never heard of PNH or this treatment, and can quickly start therapy with antibiotics.

So modifications to the eculizumab monoclonal antibody led to ravulizumab, which has a longer half-life with more sustained high levels of C5 inhibition. There were two studies that were done on this drug, one in complement inhibitor–naive patients, and the other in patients who were on eculizumab. And that one compared to ravulizumab to eculizumab. Both of these studies were noninferiority studies, and both showed that ravulizumab was noninferior to eculizumab across all the endpoints that were studied. We can't claim it's superior, but we do know that it's certainly much more convenient to give this drug every 8 weeks than every 2 weeks. And because it provides a higher level of C5 inhibition over that period, you probably get less breakthrough hemolysis events. You still need to vaccinate against meningococcus with this antibody, and that's true for all the complement inhibitors.

Finally, crovalimab is out there. It is a novel anti-C5 monoclonal antibody that was engineered using what was called smart technology so it bypasses the liver and has a long half-life, and you can use small volumes. Because of that, you can give it subcutaneously in smaller volumes. And the hope was that you could actually take this home and administer at home for patients. Three studies led to its FDA approval last year, COMMODORE 1, COMMODORE 2, and COMMODORE 3, which you can see were either in C5 treated or in C5 inhibitor–naive patients. This drug also was done in patients up to above the age of 13, or it is FDA approved for above the age of

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13, so some pediatric patients.

One thing we learned when giving this there was an overlap when you had the C5 inhibitor still in your system and crovalimab. And because they bind to different epitopes, you could get these immune complexes that formed, which were very worrisome. Clinically, however, they just led to rash, which was limited to a few weeks, and outside of a little bit of itching, really did not cause any clinical problems, including no renal failure or other problems like that.

Now, despite all of these C5 inhibitors being very, very good drugs, and its survival improving markedly, and symptoms improving, patients were still having suboptimal responses. Most patients continue to show a low level of hemolysis, 25 to 35% still required red cell transfusions. And what we learned is that eculizumab, though it blocks C5 and prevents its cleavage, C3 was still being cleaved and activated, and C3 fragments were coding these PNH red cells because they had no way of removing them, because of the lack of the GPI active proteins. And that would eventually lead to uptake in the spleen and liver and the reticuloendothelial cells, and extravascular hemolysis to various levels in patients. Some of these patients, it could be severe enough. And we estimated that 5 to 20% of patients on C5 inhibitors had a suboptimal response due to most often C3 coding of the red cells.

This led then to studies of the proximal pathway of C3 factor D and factor B inhibition, as shown in this slide. And we now have three FDA approved drugs that target those three factors. Pegcetacoplan was the first, which was FDA approved in May of 2021. It is given subcutaneously twice a week, for all adult patients with PNH. Danicopan is a factor D inhibitor that's given orally three times a day, and it has to be given with a C5 inhibitor, so you are not getting rid of your IV therapy with this. Iptacopan is an oral factor B inhibitor that is used as a monotherapy, and it's given twice a day.

So let's talk first about pegcetacoplan. The PEGASUS was the phase 3 registry trial comparing pegcetacoplan to eculizumab in patients who were on eculizumab with a hemoglobin less than 10.5, a suboptimal responder, in a sense. During the first 4 weeks, all patients received both drugs together. So you see that rise in hemoglobin from 8.7 to 11.8-11.9 range. And then they were randomized to receiving either eculizumab alone or pegcetacoplan alone. And the primary endpoint of this was a change in the hemoglobin at week 16. And what you see here is that patients on eculizumab therapy fell right back to their baseline hemoglobin, whereas those in pegcetacoplan maintain this effect of staying in the close to normal range around 12.

There were a few adverse events, including injection site reaction, since it's a sub-q drug, diarrhea which was mild and self-limited, and some episodes of breakthrough hemolysis, although the number of breakthrough hemolysis events were actually higher in the eculizumab arm than in the pegcetacoplan arm. Again, safety outcomes are shown here. The breakthrough hemolysis occurred in 13 patients, 9 who were on the eculizumab arm, 4 on the pegcetacoplan arm, 3 patients had to discontinue it because we didn't know what to do if they had hemolytic anemia. We put them back on the C5 inhibitor. We now have a study out there showing that if you give an extra dose of pegcetacoplan, you can abrogate that breakthrough hemolysis.

PRINCE was a study done with the same drug in naive patients, complement inhibitor–naive patients. And you show here again that rise in hemoglobin to above 12 with a low LDH. And these patients' reticulocyte counts fell. Everything looked very good for this drug. So it's now FDA approved and given primarily to patients with extravascular hemolysis or suboptimal response to C5 inhibitors, but it can be used up front in some patients.

Danicopan is the oral factor D inhibitor that was FDA approved in May of last year, given orally three times a day with a C5 inhibitor. They did not want to try it without that. In part, they were worried about breakthrough hemolysis on just a single agent. And you see here again, the primary endpoints were co-endpoints of either a hemoglobin rise of greater than 2 g/dL, or a hemoglobin rise to above 12. And you see that that did occur in the majority of patients treated with danicopan plus a C5 inhibitor as compared to placebo. Again, dramatically, on this graph, where you see patients treated with danicopan plus the C5 inhibitor versus those just on the C5 inhibitor alone in the green line, that patients' hemoglobin levels rose to above 10 when they were below 8 in the baseline for these patients, and this was sustained out greater than 48 weeks. So it provides another option for treating patients.

And finally, we have iptacopan, which is a factor B inhibitor, oral monotherapy given twice a day to adult patients with PNH. APPLY and APPOINT were the two studies that were done, phase 3 studies. APPLY is in C5 inhibitor-treated patients. APPOINT is in treatment-naive patients. Again, co-endpoints of a rise of greater than 2 or hemoglobin level greater than 12. And these were both met in the significant majority of patients compared to those that stayed on the C5 inhibitor alone. There was marked improvements in transfusion and independence, fatigue compared to standard of care. And this is just the APPOINT study showing the same thing, hemoglobin

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levels rising to near normal levels.

We have data out for the same drug for over 48 weeks, showing, again, a sustained response to the iptacopan given orally, it doesn't go away. And the side effects, again, have been very good with this drug. Everything looking good.

The one thing I will say about iptacopan, and we'll have to wait with more time to see if this true, but the number of breakthrough hemolysis events, that is, you get a complement-activated event, like an infection, and you can get breakthrough hemolysis with all these drugs, but it seemed to be lower with iptacopan. There is no head-to-head study to compare these, but historically, the number of breakthrough events with iptacopan seemed to be a little bit lower.

With all these patients, we have to watch out for hyperlipidemia. It's not clear why they can get that. It's mild, and in most patients, it was managed easily with either diet modifications and/or with increasing their dose of statins. All patients have to be vaccinated against meningitis, as mentioned. In addition, if you're on a proximal inhibitor, you should receive the pneumococcal vaccine and possibly the Hemophilus influenza vaccine.

Vaccines can activate complements, so we have to be careful with that if you're not on a C5 inhibitor or a proximal inhibitor. And it's been recommended in some cases that we actually give prophylactic antibiotics, start the treatment with the complement inhibitor, and then vaccinate right away, so we can avoid that hemolysis. Infection should all receive immediate antibiotics. Stopping complement inhibitors is not recommended, even if you have an infection. The FDA label, for example, says, hey, if you get a serious infection, consider stopping it. But the reality is, if you do that, you're going to see a lot more hemolysis. And extravascular hemolysis is a complication that we just talked about, and in those cases, we should probably switch to a proximal inhibitor. Breakthrough hemolysis is usually related to complement-activating events such as infection or bacteria. Except for pegcetacoplan, we don't really have clinical guidelines to help do this. There is recommendations from an expert panel, although it is a little bit scattered all over the place in terms of what they recommend. I recommend continuing the current complement inhibitor they're on. You obviously treat the infection or whatever complement event there is – complement-activated event there is and consider giving an extra dose of whatever they're on, which is what we do with pegcetacoplan. Thrombotic events need to be treated with thrombolytics and anticoagulants as indicated. You have to be careful for bleeding. There is no data if you are on a complement inhibitor, if switching to another component inhibitor will help prevent another clot.

How to choose which one to use is not easy. It takes somebody who knows all the drugs well to discuss with the patient, asking the patients what they would prefer is one method. But obviously there are side effects to all these drugs, and you need to know which one is best for the patient. We have to consider side effects, rate of thromboses, the cost of these drugs, they're all very expensive, whether insurance will cover them, and we have to make sure the patients can be compliant, especially if they're on an oral drug, because if you stop or miss a dose, you can quickly go into a hemolytic flare. All these drugs are well tolerated, with few side effects. And choosing the right therapy might take consultation with a center that specializes in PNH.

We have a couple of cases that we'll present in our grand rounds series to talk about how to treat patients with PNH and give you examples. And I think they're very interesting.

Patient C is a 41-year-old female. She initially presented 20 years ago with shortness of breath and heavy bleeding from her menses. She was found to be markedly pancytopenic, and she had a workup including a bone marrow biopsy, which revealed aplastic anemia. She was treated with ATG and cyclosporine, and had a complete count recovery and a bone marrow biopsy a year later was normocellular, so she had a wonderful response. She was followed until about 8 years later, when she began having fatigue. She came in and had marked anemia with a hemoglobin of 5.7. Other counts were a little bit low, but not bad. She was referred to her local hematologist who did a bone marrow biopsy and she was cellular. She did not have aplastic anemia. She did not have MDS. And he was smart enough to do a peripheral blood flow cytometry for PNH, which was positive. She got started on eculizumab in September of 2016, and did okay. Her hemoglobin rose to 9.4 but it wasn't great, and she was still having fatigue, and her LDH was still not completely normal. The local hematologist actually referred her for a bone marrow transplant, and the transplant team said she was not felt to be a candidate. I don't know the reasons why. She was still not feeling great, and she was requiring transfusions about every 3 months, despite increasing the dose of eculizumab. And she eventually got switched to ravulizumab in December of 2020. And about a year later, she was still not having a great response, and was referred to our center. Her hemoglobin was 7.6 as you see there. White cells and platelets were normal. Her LDH was just slightly elevated. Her bilirubin was markedly elevated, and she had a retic count that was sky high – or a retic percentage that was sky high. At that time, we felt she was a suboptimal responder, likely due to the C3 coating of

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her red cells and extravascular hemolysis. We started her on iptacopan in January of 2024, shortly after it got FDA approved. We last saw her last October. Actually, we've seen her since then. She has absolutely no complaints. Her hemoglobin is now normal at 14.8. Her LDH is normal, her retic count is normal, her bilirubin is normal, and she feels very well with stable blood counts. This is a great example of what's called a suboptimal responder to C5 inhibitor therapy due to extravascular hemolysis, who then responds to a proximal complement inhibitor.

And she still feels well with very stable blood counts, and is doing quite well.

With that, I would like to thank you so much for your attention for this interesting activity, and I hope you learned a good bit.

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