Bölüm
29

PRİMER AMİLOİDOZDA TEDAVİ

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GİRİŞ

Amiloidoz, yanlış katlanmış proteinlerin doku infiltrasyonu ile karakterize heterojen bir hastalık grubudur. Amiloidoz birikintilerinin protein altbirim bileşimine göre sınıflandırılmaktadırlar. Bu bölümde plazma hücre diskrazisi olan primer sistemik hafif zincir (AL) amiloidozu tedavisi değerlendirilecektir. AL amiloidozunda tedavinin asıl amacı organ işlevini iyileştirmek ve hastanın sağkalımını uzatmaktır. Tedavi ile patolojik plazma hücreleri yok edilmesi ve dolaşımdaki serbest hafif zincirin ortadan kaldırılması hedeflenmektedir.[1] Tedavi seçimi hastanın yaşı, komorbiditeleri, organ tutulumu, tedavi amacı ve hastanın istekleri doğrultusunda düzenlenmelidir.[2]

AL AMİLOİDOZUNDA TEDAVİ SEÇENEKLERİ

AL amiloid hastası için tedaviyi değerlendirmedeki ilk adım kök hücre nakli için uygunluğunun belirlenmesidir.[3] Transplant uygunluğu hastanın yaşı (<70yaş), troponin T düzeyi (<0,06mcg/L), GFR (>40cc/dk) ve sistolik kan basıncına (>100) göre değerlendirilir. Yeni tanı alan hastaların yaş, kalp ve böbrek yetmezliği nedeni ile en fazla %25'i transplant adayı olabilmektedir.[3] Diğer hastalar ise sistemik kemoterapi tedavisi adayıdır.

AL amiloidozun tedavisi için kemoterapi kullanılmaya başlanmıası 1972'de uygulamaya başlandı.[4] AL amiloidozda kemoterapinin ilk amacı, toksisiteyi ve tedaviyle ilişkili mortaliteyi en aza indirirken, mümkün olduğu kadar çabuk yeterli ve dayanıklı bir hematolojik yanıt elde etmektir.[2] Kemoterapi uygulaması sonrası amiloid birikiminde gerileme olabileceği gösterilmiştir.[5] Sonuç olarak erken tanı koymak ve tedaviye zamanında başlamak doku organ hasarlarının daha

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bortezomib bazlı olmalıdır.[3] Kırılgan hastalar için, melfalan ve deksametazon ile oral tedavi uygundur. İkinci basamak tedavide immünomodülatör bazlı tedaviler veya daratumumab gibi yanıt oranı yüksek ajanlar akılda bulundurulmalıdır. Anti-amiloid antikorları umut vaad etmekte olup, bu hastaların gelecekteki yönetiminde potansiyel rol oynayacakları düşünülmektedir. Bu konuda daha geniş, prospektif çalışmaların yapılmasına ihtiyaç duyulmaktadır.

Anahtar Kelimeler: amiloidoz, AL, tedavi

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